

Anhang 4-G: Ergänzende Unterlagen

Subgruppenanalysen der Studien BMN 111-301 und BMN 111-206 (RCT)

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Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

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Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

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Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum	832
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Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

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Interaction	1000
Safety	
Preferred Term	1006
AE Category	1034
Interaction	1066
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Efficacy	
Annualized Growth Velocity (cm/year)	1071
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Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

Upper to Lower Body Segment Ratio	1215
Interaction	1287
Upper Arm Length to Lower Arm (Forearm) Length Ratio	1290
Upper Leg Length (Thigh) to Knee to Heel Length Ratio	1362
Upper Leg Length (Thigh) to Tibial Leg Length Ratio	1434
Arm Span to Standing Height Ratio	1506
Interaction	1578
QoL	
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Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

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Over Time by Age at Baseline: Total Score	1878
Over Time by Baseline Tanner Stage: Total Score	1888
Over Time by Baseline Height Z-score: Total Score	1896
Over Time by Baseline AGV Category: Total Score	1912
Over Time by Ethnicity: Total Score	1924
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Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

Over Time by Sex: Psychosocial Summary Score	1994
Over Time by Age at Baseline: Psychosocial Summary Score	2002
Over Time by Baseline Tanner Stage: Psychosocial Summary Score	2012
Over Time by Baseline Height Z-score: Psychosocial Summary Score	2020
Over Time by Baseline AGV Category: Psychosocial Summary Score	2036
Over Time by Ethnicity: Psychosocial Summary Score	2048
Over Time by Sex: Emotional Functioning Score	2056
Over Time by Age at Baseline: Emotional Functioning Score	2064
Over Time by Baseline Tanner Stage: Emotional Functioning Score	2074
Over Time by Baseline Height Z-score: Emotional Functioning Score	2082
Over Time by Baseline AGV Category: Emotional Functioning Score	2098
Over Time by Ethnicity: Emotional Functioning Score	2110
Over Time by Sex: Social Functioning Score	2118
Over Time by Age at Baseline: Social Functioning Score	2126
Over Time by Baseline Tanner Stage: Social Functioning Score	2136
Over Time by Baseline Height Z-score: Social Functioning Score	2144
Over Time by Baseline AGV Category: Social Functioning Score	2160
Over Time by Ethnicity: Social Functioning Score	2172
Over Time by Sex: School Functioning Score	2180
Over Time by Age at Baseline: School Functioning Score	2188
Over Time by Baseline Tanner Stage: School Functioning Score	2198
Over Time by Baseline Height Z-score: School Functioning Score	2206
Over Time by Baseline AGV Category: School Functioning Score	2222
Over Time by Ethnicity: School Functioning Score	2234
QoLISSY (caregiver-reported)	2242
Over Time by Sex: Total Score	2242
Over Time by Age at Baseline: Total Score	2248
Over Time by Baseline Tanner Stage: Total Score	2257
Over Time by Baseline Height Z-score: Total Score	2263

Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

Over Time by Baseline AGV Category: Total Score	2275
Over Time by Ethnicity: Total Score	2284
Over Time by Sex: Physical Score	2290
Over Time by Age at Baseline: Physical Score	2296
Over Time by Baseline Tanner Stage: Physical Score	2305
Over Time by Baseline Height Z-score: Physical Score	2311
Over Time by Baseline AGV Category: Physical Score	2323
Over Time by Ethnicity: Physical Score	2332
Over Time by Sex: Social Score	2338
Over Time by Age at Baseline: Social Score	2344
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Over Time by Baseline AGV Category: Social Score	2371
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Over Time by Baseline AGV Category: Emotional Score	2419
Over Time by Ethnicity: Emotional Score	2428
Over Time by Sex: Coping Score	2434
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Over Time by Baseline Height Z-score: Coping Score	2455
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Over Time by Age at Baseline: Beliefs Score	2488
Over Time by Baseline Tanner Stage: Beliefs Score	2497

Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

Over Time by Baseline Height Z-score: Beliefs Score	2503
Over Time by Baseline AGV Category: Beliefs Score	2515
Over Time by Ethnicity: Beliefs Score	2524
Over Time by Sex: Future Score	2530
Over Time by Age at Baseline: Future Score	2536
Over Time by Baseline Tanner Stage: Future Score	2545
Over Time by Baseline Height Z-score: Future Score	2551
Over Time by Baseline AGV Category: Future Score	2563
Over Time by Ethnicity: Future Score	2572
Over Time by Sex: Effects Score	2578
Over Time by Age at Baseline: Effects Score	2584
Over Time by Baseline Tanner Stage: Effects Score	2593
Over Time by Baseline Height Z-score: Effects Score	2599
Over Time by Baseline AGV Category: Effects Score	2611
Over Time by Ethnicity: Effects Score	2620
QoLISSY (self-reported)	2626
Over Time by Sex: Total Score	2626
Over Time by Age at Baseline: Total Score	2634
Over Time by Baseline Tanner Stage: Total Score	2645
Over Time by Baseline Height Z-score: Total Score	2653
Over Time by Baseline AGV Category: Total Score	2669
Over Time by Ethnicity: Total Score	2681
Over Time by Sex: Physical Score	2689
Over Time by Age at Baseline: Physical Score	2695
Over Time by Baseline Tanner Stage: Physical Score	2704
Over Time by Baseline Height Z-score: Physical Score	2710
Over Time by Baseline AGV Category: Physical Score	2722
Over Time by Ethnicity: Physical Score	2731
Over Time by Sex: Social Score	2737

Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

Over Time by Age at Baseline: Social Score	2743
Over Time by Baseline Tanner Stage: Social Score	2752
Over Time by Baseline Height Z-score: Social Score	2758
Over Time by Baseline AGV Category: Social Score	2770
Over Time by Ethnicity: Social Score	2779
Over Time by Sex: Emotional Score	2785
Over Time by Age at Baseline: Emotional Score	2791
Over Time by Baseline Tanner Stage: Emotional Score	2800
Over Time by Baseline Height Z-score: Emotional Score	2806
Over Time by Baseline AGV Category: Emotional Score	2818
Over Time by Ethnicity: Emotional Score	2827
Over Time by Sex: Coping Score	2833
Over Time by Age at Baseline: Coping Score	2839
Over Time by Baseline Tanner Stage: Coping Score	2848
Over Time by Baseline Height Z-score: Coping Score	2854
Over Time by Baseline AGV Category: Coping Score	2866
Over Time by Ethnicity: Coping Score	2875
Over Time by Sex: Beliefs Score	2881
Over Time by Age at Baseline: Beliefs Score	2887
Over Time by Baseline Tanner Stage: Beliefs Score	2896
Over Time by Baseline Height Z-score: Beliefs Score	2902
Over Time by Baseline AGV Category: Beliefs Score	2914
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Over Time by Sex: Total Score	2929
Over Time by Age at Baseline: Total Score	2935
Over Time by Baseline Tanner: Total Score	2944
Over Time by Baseline Height Z-score: Total Score	2950
Over Time by Baseline AGV Category: Total Score	2962

Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamen Zusatznutzen

Over Time by Ethnicity: Total Score	2971
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Over Time by Age at Baseline: Self Care Score	2983
Over Time by Baseline Tanner: Self Care Score	2992
Over Time by Baseline Height Z-score: Self Care Score	2998
Over Time by Baseline AGV Category: Self Care Score	3010
Over Time by Ethnicity: Self Care Score	3019
Over Time by Sex: Mobility Score	3025
Over Time by Age at Baseline: Mobility Score	3031
Over Time by Baseline Tanner: Mobility Score	3040
Over Time by Baseline Height Z-score: Mobility Score	3046
Over Time by Baseline AGV Category: Mobility Score	3058
Over Time by Ethnicity: Mobility Score	3067
Over Time by Sex: Cognitive Score	3073
Over Time by Age at Baseline: Cognitive Score	3079
Over Time by Baseline Tanner: Cognitive Score	3088
Over Time by Baseline Height Z-score: Cognitive Score	3094
Over Time by Baseline AGV Category: Cognitive Score	3106
Over Time by Ethnicity: Cognitive Score	3115
Interaction	3121
Safety	
Preferred Term	3155
AE Category	3175
Interaction	3215

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	16
Mean (SD)	-4.55 (1.65)	-4.06 (1.11)
Median	-4.78	-3.99
25th, 75th Percentile	-5.69, -3.86	-4.67, -3.25
Min, Max	-7.2, -1.9	-5.9, -2.2
Week 52		
n	13	16
Mean (SD)	-4.50 (1.33)	-4.09 (0.84)
Median	-4.29	-4.20

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.101.001_mod_sub_sex_haz_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.64, -3.43	-4.88, -3.45
Min, Max	-6.4, -2.5	-5.1, -2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.101.001_mod_sub_sex_haz_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	16
Mean (SD)	0.05 (0.72)	-0.04 (0.66)
Median	0.10	-0.11
25th, 75th Percentile	-0.43, 0.34	-0.29, 0.59
Min, Max	-1.4, 1.3	-1.3, 0.9
LS mean change from baseline (95% CI)	-0.14 (-0.38, 0.10)	0.12 (-0.10, 0.33)
Difference in LS mean change from baseline (95% CI) ^a		0.25 (-0.08, 0.59)
P-value ^b		0.1277

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.101.001_mod_sub_sex_haz_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.63 (-0.18, 1.42)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.101.001_mod_sub_sex_haz_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Female		
Baseline		
n	19	15
Mean (SD)	-4.09 (1.36)	-3.54 (0.75)
Median	-4.02	-3.67
25th, 75th Percentile	-5.41, -3.09	-4.19, -2.94
Min, Max	-6.8, -1.5	-4.8, -2.2
Week 52		
n	19	15
Mean (SD)	-4.52 (1.11)	-3.74 (0.71)
Median	-4.63	-3.85

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.54, -3.73	-4.13, -3.06
Min, Max	-6.6, -2.1	-5.4, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	19	15
Mean (SD)	-0.43 (0.49)	-0.19 (0.61)
Median	-0.32	-0.48
25th, 75th Percentile	-0.69, -0.07	-0.66, 0.38
Min, Max	-1.8, 0.2	-1.2, 0.9
LS mean change from baseline (95% CI)	-0.47 (-0.68, -0.26)	-0.15 (-0.39, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		0.32 (-0.02, 0.66)
P-value ^b		0.0612

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.101.1
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.78 (-0.04, 1.57)
P-value for interaction term, treatment * [Sex]		0.2528

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	25	20
Mean (SD)	-4.31 (1.45)	-3.74 (0.92)
Median	-4.02	-3.68
25th, 75th Percentile	-5.47, -3.31	-4.15, -3.04
Min, Max	-7.2, -1.5	-5.9, -2.5
Week 52		
n	25	20
Mean (SD)	-4.51 (1.19)	-3.79 (0.70)
Median	-4.29	-3.81

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.60, -3.43	-4.17, -3.23
Min, Max	-6.6, -2.1	-5.1, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	25	20
Mean (SD)	-0.21 (0.62)	-0.05 (0.70)
Median	-0.26	0.00
25th, 75th Percentile	-0.54, 0.04	-0.60, 0.53
Min, Max	-1.4, 1.3	-1.3, 0.9
LS mean change from baseline (95% CI)	-0.26 (-0.44, -0.07)	0.01 (-0.20, 0.22)
Difference in LS mean change from baseline (95% CI) ^a		0.27 (-0.02, 0.55)
P-value ^b		0.0629

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.60 (-0.03, 1.22)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Non-White		
Baseline		
n	7	11
Mean (SD)	-4.17 (1.68)	-3.94 (1.11)
Median	-4.78	-4.08
25th, 75th Percentile	-5.03, -2.30	-4.80, -3.17
Min, Max	-6.7, -2.0	-5.8, -2.2
Week 52		
n	7	11
Mean (SD)	-4.52 (1.25)	-4.16 (0.90)
Median	-4.68	-4.20

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.37, -3.73	-4.97, -3.44
Min, Max	-6.4, -2.5	-5.4, -2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.2.101.2
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	11
Mean (SD)	-0.35 (0.69)	-0.22 (0.49)
Median	-0.28	-0.27
25th, 75th Percentile	-0.51, 0.10	-0.58, -0.10
Min, Max	-1.8, 0.3	-1.0, 0.8
LS mean change from baseline (95% CI)	-0.31 (-0.62, 0.01)	-0.25 (-0.50, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		0.06 (-0.37, 0.48)
P-value ^b		0.7738

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Table 14.2.1.2.101.2
 Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.16 (-0.93, 1.25)
P-value for interaction term,treatment * [Ethnicity]		0.7009

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	-4.99 (1.47)	-4.13 (0.80)
Median	-4.77	-4.05
25th, 75th Percentile	-6.21, -3.77	-4.39, -3.59
Min, Max	-6.7, -3.7	-5.8, -3.2
Week 52		
n	4	8
Mean (SD)	-5.05 (1.17)	-4.26 (0.49)
Median	-4.97	-4.19

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.101.3
 Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-6.02, -4.08	-4.62, -4.01
Min, Max	-6.4, -3.9	-5.0, -3.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.101.003_mod_sub_strat_haz_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	-0.06 (0.33)	-0.13 (0.52)
Median	-0.07	-0.23
25th, 75th Percentile	-0.30, 0.19	-0.46, 0.14
Min, Max	-0.4, 0.3	-0.8, 0.8
LS mean change from baseline (95% CI)	-0.23 (-0.73, 0.27)	-0.04 (-0.38, 0.29)
Difference in LS mean change from baseline (95% CI) ^a		0.19 (-0.46, 0.83)
P-value ^b		0.5014

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.52 (-0.95, 1.95)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	-5.17 (1.10)	-4.43 (0.85)
Median	-4.99	-4.36
25th, 75th Percentile	-5.50, -4.79	-4.80, -4.12
Min, Max	-7.2, -3.0	-5.9, -3.1
Week 52		
n	12	7
Mean (SD)	-5.06 (1.01)	-4.00 (0.95)
Median	-5.17	-3.85

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.82, -4.46	-5.10, -3.46
Min, Max	-6.6, -2.8	-5.4, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	0.11 (0.54)	0.43 (0.49)
Median	-0.01	0.48
25th, 75th Percentile	-0.33, 0.21	0.34, 0.84
Min, Max	-0.4, 1.3	-0.6, 0.9
LS mean change from baseline (95% CI)	0.11 (-0.15, 0.36)	0.44 (0.10, 0.78)
Difference in LS mean change from baseline (95% CI) ^a		0.33 (-0.11, 0.77)
P-value ^b		0.1309

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Table 14.2.1.2.101.3
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.84 (-0.25, 1.90)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.5259

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	7
Mean (SD)	-5.17 (1.07)	-4.44 (1.27)
Median	-4.97	-4.19
25th, 75th Percentile	-5.64, -4.51	-5.79, -4.08
Min, Max	-7.2, -3.7	-5.9, -2.2
Week 52		
n	12	7
Mean (SD)	-5.14 (0.91)	-4.23 (1.01)
Median	-5.17	-4.18

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.84, -4.26	-5.10, -3.66
Min, Max	-6.6, -3.9	-5.4, -2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	0.03 (0.58)	0.21 (0.55)
Median	-0.12	0.34
25th, 75th Percentile	-0.38, 0.16	-0.31, 0.82
Min, Max	-0.5, 1.3	-0.6, 0.8
LS mean change from baseline (95% CI)	0.03 (-0.27, 0.33)	0.21 (-0.19, 0.61)
Difference in LS mean change from baseline (95% CI) ^a		0.18 (-0.36, 0.72)
P-value ^b		0.4760

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.41 (-0.70, 1.50)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	20	24
Mean (SD)	-3.74 (1.45)	-3.62 (0.81)
Median	-3.63	-3.58
25th, 75th Percentile	-4.95, -2.60	-4.14, -3.04
Min, Max	-6.7, -1.5	-5.7, -2.2
Week 52		
n	20	24
Mean (SD)	-4.14 (1.19)	-3.83 (0.71)
Median	-3.77	-3.88

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Table 14.2.1.2.101.4
 Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.13, -3.34	-4.33, -3.23
Min, Max	-6.4, -2.1	-5.0, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

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Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	20	24
Mean (SD)	-0.40 (0.61)	-0.21 (0.63)
Median	-0.31	-0.23
25th, 75th Percentile	-0.81, 0.02	-0.71, 0.22
Min, Max	-1.8, 0.6	-1.3, 0.9
LS mean change from baseline (95% CI)	-0.43 (-0.63, -0.22)	-0.18 (-0.37, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		0.25 (-0.04, 0.53)
P-value ^b		0.0844

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.101.004_mod_sub_agv_haz_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.4
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.56 (-0.07, 1.18)
P-value for interaction term, treatment * [Baseline AGV]		0.7164

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	18	13
Mean (SD)	-5.32 (0.91)	-4.70 (0.69)
Median	-5.26	-4.46
25th, 75th Percentile	-5.69, -4.80	-4.83, -4.19
Min, Max	-7.2, -4.0	-5.9, -4.0
Week 52		
n	18	13
Mean (SD)	-5.30 (0.85)	-4.41 (0.65)
Median	-5.45	-4.20

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
 Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-5.87, -4.68	-5.00, -3.87
Min, Max	-6.6, -3.4	-5.4, -3.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	13
Mean (SD)	0.03 (0.52)	0.29 (0.50)
Median	-0.05	0.38
25th, 75th Percentile	-0.34, 0.22	-0.18, 0.72
Min, Max	-0.6, 1.3	-0.6, 0.9
LS mean change from baseline (95% CI)	-0.02 (-0.24, 0.21)	0.35 (0.08, 0.63)
Difference in LS mean change from baseline (95% CI) ^a		0.37 (-0.03, 0.77)
P-value ^b		0.0698

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
 Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.92 (-0.07, 1.90)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	18
Mean (SD)	-2.93 (0.81)	-3.17 (0.55)
Median	-3.04	-3.23
25th, 75th Percentile	-3.69, -2.30	-3.67, -2.72
Min, Max	-4.0, -1.5	-3.9, -2.2
Week 52		
n	14	18
Mean (SD)	-3.51 (0.70)	-3.57 (0.69)
Median	-3.58	-3.53

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	-3.86, -3.32	-4.13, -2.97
Min, Max	-4.8, -2.1	-4.7, -2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	18
Mean (SD)	-0.58 (0.60)	-0.40 (0.57)
Median	-0.47	-0.49
25th, 75th Percentile	-1.00, -0.18	-0.77, -0.03
Min, Max	-1.8, 0.3	-1.3, 0.9
LS mean change from baseline (95% CI)	-0.51 (-0.70, -0.31)	-0.46 (-0.63, -0.29)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (-0.22, 0.31)
P-value ^b		0.7236

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.101.5
 Analysis of Covariance of Height Z-Score at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Height Z-Score	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.14 (-0.62, 0.89)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.0776

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.1
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	-5.20 (1.50)	-4.57 (0.99)
Median	-5.12	-4.36
25th, 75th Percentile	-6.74, -3.86	-5.79, -4.08
Min, Max	-7.2, -3.0	-5.9, -3.2
Week 52		
n	7	7
Mean (SD)	-4.83 (1.24)	-4.23 (0.73)
Median	-4.68	-4.18

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.1
 Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-5.92, -4.12	-4.97, -3.46
Min, Max	-6.4, -2.8	-5.1, -3.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.1
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	0.37 (0.60)	0.34 (0.54)
Median	0.20	0.46
25th, 75th Percentile	0.04, 1.00	-0.27, 0.84
Min, Max	-0.4, 1.3	-0.3, 0.9
LS mean change from baseline (95% CI)	0.23 (-0.06, 0.51)	0.48 (0.20, 0.77)
Difference in LS mean change from baseline (95% CI) ^a		0.26 (-0.15, 0.67)
P-value ^b		0.1876

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.1
 Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.83 (-0.39, 1.99)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.1
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	-5.07 (0.88)	-4.00 (0.55)
Median	-4.87	-4.11
25th, 75th Percentile	-5.47, -4.80	-4.36, -3.59
Min, Max	-6.8, -3.7	-4.8, -3.1
Week 52		
n	9	8
Mean (SD)	-5.23 (0.83)	-4.05 (0.76)
Median	-5.22	-4.01

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.1.2.102.1
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-5.76, -4.89	-4.33, -3.86
Min, Max	-6.6, -3.9	-5.4, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.1
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	-0.16 (0.21)	-0.05 (0.56)
Median	-0.18	0.08
25th, 75th Percentile	-0.34, -0.02	-0.62, 0.43
Min, Max	-0.4, 0.2	-0.8, 0.6
LS mean change from baseline (95% CI)	-0.23 (-0.60, 0.14)	0.03 (-0.37, 0.43)
Difference in LS mean change from baseline (95% CI) ^a		0.26 (-0.35, 0.87)
P-value ^b		0.3734

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.1.2.102.1
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.58 (-0.68, 1.82)
P-value for interaction term, treatment * [Sex]		0.3017

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	-5.05 (1.19)	-4.17 (0.85)
Median	-5.12	-4.15
25th, 75th Percentile	-5.53, -4.23	-4.41, -3.59
Min, Max	-7.2, -3.0	-5.9, -3.1
Week 52		
n	13	8
Mean (SD)	-5.00 (1.05)	-3.90 (0.72)
Median	-5.22	-3.86

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-5.76, -4.23	-4.30, -3.56
Min, Max	-6.6, -2.8	-5.1, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 ($\geq 24 < 60$ months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	0.05 (0.54)	0.27 (0.64)
Median	-0.07	0.47
25th, 75th Percentile	-0.34, 0.20	-0.16, 0.72
Min, Max	-0.4, 1.3	-0.8, 0.9
LS mean change from baseline (95% CI)	0.02 (-0.19, 0.22)	0.33 (0.06, 0.60)
Difference in LS mean change from baseline (95% CI) ^a		0.31 (-0.04, 0.67)
P-value ^b		0.0768

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.94 (-0.10, 1.96)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	-5.46 (1.10)	-4.38 (0.80)
Median	-4.87	-4.26
25th, 75th Percentile	-6.74, -4.78	-4.80, -4.02
Min, Max	-6.7, -4.8	-5.8, -3.2
Week 52		
n	3	7
Mean (SD)	-5.32 (0.93)	-4.41 (0.67)
Median	-4.89	-4.20

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-6.39, -4.68	-4.97, -3.89
Min, Max	-6.4, -4.7	-5.4, -3.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	0.14 (0.18)	-0.03 (0.47)
Median	0.10	-0.19
25th, 75th Percentile	-0.02, 0.34	-0.27, 0.38
Min, Max	0.0, 0.3	-0.6, 0.8
LS mean change from baseline (95% CI)	1.15 (-0.12, 2.42)	-0.46 (-1.04, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		-1.61 (-3.40, 0.18)
P-value ^b		0.0646

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Table 14.2.1.2.102.2
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-7.76 (-15.49, 0.40)
P-value for interaction term, treatment * [Ethnicity]		0.8997

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

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Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.102.002_mod_sub_eth_haz_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	-4.99 (1.47)	-4.13 (0.80)
Median	-4.77	-4.05
25th, 75th Percentile	-6.21, -3.77	-4.39, -3.59
Min, Max	-6.7, -3.7	-5.8, -3.2
Week 52		
n	4	8
Mean (SD)	-5.05 (1.17)	-4.26 (0.49)
Median	-4.97	-4.19

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-6.02, -4.08	-4.62, -4.01
Min, Max	-6.4, -3.9	-5.0, -3.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	-0.06 (0.33)	-0.13 (0.52)
Median	-0.07	-0.23
25th, 75th Percentile	-0.30, 0.19	-0.46, 0.14
Min, Max	-0.4, 0.3	-0.8, 0.8
LS mean change from baseline (95% CI)	-0.23 (-0.73, 0.27)	-0.04 (-0.38, 0.29)
Difference in LS mean change from baseline (95% CI) ^a		0.19 (-0.46, 0.83)
P-value ^b		0.5014

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.52 (-0.95, 1.95)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	-5.17 (1.10)	-4.43 (0.85)
Median	-4.99	-4.36
25th, 75th Percentile	-5.50, -4.79	-4.80, -4.12
Min, Max	-7.2, -3.0	-5.9, -3.1
Week 52		
n	12	7
Mean (SD)	-5.06 (1.01)	-4.00 (0.95)
Median	-5.17	-3.85

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-5.82, -4.46	-5.10, -3.46
Min, Max	-6.6, -2.8	-5.4, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	0.11 (0.54)	0.43 (0.49)
Median	-0.01	0.48
25th, 75th Percentile	-0.33, 0.21	0.34, 0.84
Min, Max	-0.4, 1.3	-0.6, 0.9
LS mean change from baseline (95% CI)	0.11 (-0.15, 0.36)	0.44 (0.10, 0.78)
Difference in LS mean change from baseline (95% CI) ^a		0.33 (-0.11, 0.77)
P-value ^b		0.1309

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.3
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.84 (-0.25, 1.90)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.5259

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline height z-score, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
Baseline		
n	11	6
Mean (SD)	-5.11 (1.10)	-4.82 (0.85)
Median	-4.82	-4.50
25th, 75th Percentile	-5.47, -4.23	-5.79, -4.12
Min, Max	-7.2, -3.7	-5.9, -4.1
Week 52		
n	11	6
Mean (SD)	-5.03 (0.86)	-4.52 (0.72)
Median	-5.12	-4.57

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
 Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-5.76, -4.23	-5.10, -3.85
Min, Max	-6.6, -3.9	-5.4, -3.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.1.2.102.4
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	0.08 (0.58)	0.30 (0.55)
Median	-0.07	0.40
25th, 75th Percentile	-0.35, 0.22	-0.10, 0.82
Min, Max	-0.4, 1.3	-0.6, 0.8
LS mean change from baseline (95% CI)	0.12 (-0.18, 0.42)	0.22 (-0.19, 0.64)
Difference in LS mean change from baseline (95% CI) ^a		0.10 (-0.43, 0.64)
P-value ^b		0.6773

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
 Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.24 (-0.88, 1.36)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	-5.17 (1.38)	-3.90 (0.55)
Median	-5.53	-4.02
25th, 75th Percentile	-5.69, -4.87	-4.36, -3.47
Min, Max	-6.7, -3.0	-4.5, -3.1
Week 52		
n	5	9
Mean (SD)	-5.12 (1.40)	-3.88 (0.63)
Median	-5.64	-3.89

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.002.102.004_mod_sub_agv_haz_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
 Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	-5.87, -4.89	-4.20, -3.46
Min, Max	-6.4, -2.8	-4.8, -2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	0.04 (0.26)	0.02 (0.58)
Median	0.04	-0.19
25th, 75th Percentile	-0.02, 0.20	-0.27, 0.48
Min, Max	-0.3, 0.3	-0.8, 0.9
LS mean change from baseline (95% CI)	-0.31 (-0.88, 0.27)	0.22 (-0.17, 0.61)
Difference in LS mean change from baseline (95% CI) ^a		0.53 (-0.27, 1.32)
P-value ^b		0.1608

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.1.2.102.4
Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Height Z-Score	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		1.26 (-0.48, 2.92)
P-value for interaction term, treatment * [Baseline AGV]		0.3274

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline AGV and baseline height Z-Score.

For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	16
Mean (SD)	8.20 (7.46)	10.59 (7.79)
Median	5.14	6.16
25th, 75th Percentile	4.00, 10.96	4.32, 18.10
Min, Max	2.5, 29.7	2.7, 25.3
Week 52		
n	13	16
Mean (SD)	6.73 (1.53)	8.08 (1.86)
Median	6.37	7.25

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
25th, 75th Percentile	5.67, 7.33	6.88, 9.32
Min, Max	4.8, 9.3	5.8, 11.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	16
Mean (SD)	-1.47 (6.48)	-2.51 (6.33)
Median	0.89	0.85
25th, 75th Percentile	-2.13, 1.51	-7.23, 2.41
Min, Max	-20.4, 4.3	-14.6, 4.3
LS mean change from baseline (95% CI)	-2.58 (-3.10, -2.05)	-1.60 (-2.07, -1.13)
Difference in LS mean change from baseline (95% CI) ^a		0.98 (0.26, 1.70)
P-value ^b		0.0100

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		1.10 (0.26, 1.92)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
Female		
Baseline		
n	19	15
Mean (SD)	10.56 (7.98)	10.79 (7.22)
Median	7.14	7.29
25th, 75th Percentile	3.82, 16.38	5.33, 16.41
Min, Max	0.3, 24.3	0.6, 22.9
Week 52		
n	19	15
Mean (SD)	7.77 (2.87)	8.08 (3.11)
Median	7.04	8.15

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
25th, 75th Percentile	5.41, 10.21	5.04, 11.28
Min, Max	4.0, 12.3	2.9, 12.6

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
Change from baseline		
n	19	15
Mean (SD)	-2.80 (6.38)	-2.72 (5.31)
Median	-2.78	-2.25
25th, 75th Percentile	-6.72, 1.53	-5.44, 0.45
Min, Max	-14.9, 7.1	-11.3, 6.4
LS mean change from baseline (95% CI)	-3.20 (-3.87, -2.54)	-2.20 (-2.96, -1.45)
Difference in LS mean change from baseline (95% CI) ^a		1.00 (-0.04, 2.04)
P-value ^b		0.0597

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		0.73 (-0.03, 1.47)
P-value for interaction term, treatment * [Sex]		0.8743

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, and treatment and sex interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	25	20
Mean (SD)	9.86 (8.03)	12.12 (7.57)
Median	5.51	12.81
25th, 75th Percentile	3.82, 15.98	5.37, 18.10
Min, Max	0.3, 29.7	0.6, 25.3
Week 52		
n	25	20
Mean (SD)	7.46 (2.45)	8.71 (2.51)
Median	6.79	8.60

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	5.57, 9.34	7.13, 10.81
Min, Max	4.0, 12.3	2.9, 12.6

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	25	20
Mean (SD)	-2.41 (6.32)	-3.41 (6.17)
Median	0.07	-3.36
25th, 75th Percentile	-4.85, 1.53	-7.23, 2.48
Min, Max	-20.4, 5.8	-14.6, 6.4
LS mean change from baseline (95% CI)	-3.23 (-3.74, -2.72)	-2.38 (-2.94, -1.81)
Difference in LS mean change from baseline (95% CI) ^a		0.85 (0.09, 1.62)
P-value ^b		0.0302

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.69 (0.07, 1.30)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
Non-White		
Baseline		
n	7	11
Mean (SD)	8.67 (7.10)	8.08 (6.59)
Median	5.16	5.42
25th, 75th Percentile	4.83, 10.96	4.20, 7.29
Min, Max	3.3, 23.7	3.0, 22.3
Week 52		
n	7	11
Mean (SD)	6.96 (2.54)	6.92 (2.11)
Median	5.69	7.00

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	5.45, 8.85	5.81, 7.26
Min, Max	4.8, 11.9	3.4, 11.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	11
Mean (SD)	-1.71 (6.93)	-1.16 (4.88)
Median	0.29	0.45
25th, 75th Percentile	-5.28, 1.51	-2.25, 1.84
Min, Max	-14.9, 7.1	-11.0, 2.5
LS mean change from baseline (95% CI)	-1.41 (-2.32, -0.51)	-1.35 (-2.05, -0.64)
Difference in LS mean change from baseline (95% CI) ^a		0.06 (-1.14, 1.26)
P-value ^b		0.9075

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.2
 Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity	Placebo (N=32)	Vosoritide (N=32)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		0.06 (-0.99, 1.11)
P-value for interaction term, treatment * [Ethnicity]		0.8446

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	5.53 (1.60)	5.52 (1.07)
Median	4.98	5.37
25th, 75th Percentile	4.42, 6.65	4.64, 6.31
Min, Max	4.4, 7.8	4.2, 7.3
Week 52		
n	4	8
Mean (SD)	5.62 (0.23)	5.76 (1.51)
Median	5.62	5.95

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	5.46, 5.79	4.89, 7.14
Min, Max	5.3, 5.9	2.9, 7.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	0.09 (1.60)	0.25 (2.01)
Median	0.48	1.03
25th, 75th Percentile	-1.03, 1.21	-1.42, 1.75
Min, Max	-2.1, 1.5	-3.0, 2.3
LS mean change from baseline (95% CI)	-0.13 (-1.67, 1.41)	0.35 (-0.72, 1.43)
Difference in LS mean change from baseline (95% CI) ^a		0.48 (-1.42, 2.39)
P-value ^b		0.5684

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.38 (-0.89, 1.63)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	3.76 (1.67)	3.85 (1.87)
Median	3.72	4.10
25th, 75th Percentile	2.83, 4.70	2.71, 5.41
Min, Max	0.3, 7.1	0.6, 5.8
Week 52		
n	12	7
Mean (SD)	5.40 (1.00)	7.01 (1.77)
Median	5.43	7.01

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	4.45, 6.15	6.76, 8.15
Min, Max	4.0, 7.3	3.4, 8.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	1.64 (2.12)	3.16 (1.86)
Median	1.39	2.78
25th, 75th Percentile	0.59, 3.05	2.31, 4.30
Min, Max	-2.8, 5.8	0.3, 6.4
LS mean change from baseline (95% CI)	1.64 (0.82, 2.47)	3.15 (2.07, 4.23)
Difference in LS mean change from baseline (95% CI) ^a		1.51 (0.14, 2.87)
P-value ^b		0.0326

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.03.002.101.003_mod_sub_strat_agv_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		1.13 (0.09, 2.14)
P-value for interaction term, treatment * [[Cohort 1]Age stratum]		0.2855

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	7
Mean (SD)	3.25 (1.16)	3.28 (1.33)
Median	3.48	3.88
25th, 75th Percentile	2.63, 4.14	2.71, 4.20
Min, Max	0.3, 4.5	0.6, 4.4
Week 52		
n	12	7
Mean (SD)	5.55 (1.00)	6.06 (1.27)
Median	5.63	6.40

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	4.64, 6.15	5.81, 7.01
Min, Max	4.0, 7.3	3.4, 7.0

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

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Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	2.30 (1.66)	2.78 (2.00)
Median	1.56	2.52
25th, 75th Percentile	1.12, 3.41	1.61, 4.30
Min, Max	0.2, 5.8	0.3, 6.4
LS mean change from baseline (95% CI)	2.44 (1.76, 3.12)	2.55 (1.64, 3.46)
Difference in LS mean change from baseline (95% CI) ^a		0.11 (-1.05, 1.28)
P-value ^b		0.8389

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.10 (-0.89, 1.09)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	20	24
Mean (SD)	13.42 (7.50)	12.85 (7.02)
Median	12.71	12.81
25th, 75th Percentile	6.32, 16.92	5.89, 19.19
Min, Max	4.8, 29.7	4.9, 25.3
Week 52		
n	20	24
Mean (SD)	8.42 (2.42)	8.67 (2.47)
Median	9.08	8.60

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	6.03, 10.07	7.20, 10.81
Min, Max	4.4, 12.3	2.9, 12.6

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	20	24
Mean (SD)	-4.99 (6.57)	-4.18 (5.56)
Median	-3.57	-3.36
25th, 75th Percentile	-6.99, -0.87	-8.97, 0.27
Min, Max	-20.4, 7.1	-14.6, 3.3
LS mean change from baseline (95% CI)	-5.01 (-5.59, -4.42)	-4.17 (-4.70, -3.64)
Difference in LS mean change from baseline (95% CI) ^a		0.84 (0.04, 1.63)
P-value ^b		0.0400

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.4
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.66 (0.03, 1.29)
P-value for interaction term, treatment * [Baseline AGV]		0.6683

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	18	13
Mean (SD)	5.63 (4.18)	5.72 (4.05)
Median	4.14	5.41
25th, 75th Percentile	3.02, 7.14	4.10, 6.68
Min, Max	0.3, 16.0	0.6, 17.7
Week 52		
n	18	13
Mean (SD)	6.00 (1.47)	7.02 (2.00)
Median	5.76	7.01

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	4.77, 6.79	6.09, 7.26
Min, Max	4.0, 9.3	3.4, 11.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and height z-score interaction.

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Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	13
Mean (SD)	0.38 (3.34)	1.31 (3.00)
Median	1.12	1.65
25th, 75th Percentile	-2.13, 2.79	0.34, 2.66
Min, Max	-6.7, 5.8	-5.7, 6.4
LS mean change from baseline (95% CI)	0.31 (-0.28, 0.90)	1.40 (0.70, 2.10)
Difference in LS mean change from baseline (95% CI) ^a		1.09 (0.12, 2.05)
P-value ^b		0.0289

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

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Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.95 (0.10, 1.79)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and height z-score interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	18
Mean (SD)	14.71 (8.37)	14.27 (7.23)
Median	15.25	14.13
25th, 75th Percentile	5.14, 23.21	5.94, 20.80
Min, Max	4.4, 29.7	3.9, 25.3
Week 52		
n	14	18
Mean (SD)	9.07 (2.38)	8.84 (2.59)
Median	9.37	9.09

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	6.37, 11.09	7.25, 10.84
Min, Max	5.3, 12.3	2.9, 12.6

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	18
Mean (SD)	-5.64 (7.72)	-5.44 (5.67)
Median	-4.32	-4.83
25th, 75th Percentile	-13.27, 0.89	-11.04, -0.60
Min, Max	-20.4, 7.1	-14.6, 2.8
LS mean change from baseline (95% CI)	-5.67 (-6.29, -5.05)	-5.41 (-5.95, -4.87)
Difference in LS mean change from baseline (95% CI) ^a		0.26 (-0.59, 1.11)
P-value ^b		0.5300

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.101.5
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Annualized Growth Velocity (cm/year)	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.24 (-0.51, 0.98)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.2061

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	4.70 (1.69)	4.45 (0.94)
Median	4.46	4.43
25th, 75th Percentile	3.34, 5.51	4.10, 5.41
Min, Max	2.6, 7.8	2.7, 5.4
Week 52		
n	7	7
Mean (SD)	5.89 (0.82)	6.97 (0.95)
Median	5.67	7.01

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.1
 Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	5.35, 6.37	6.09, 7.26
Min, Max	4.8, 7.3	5.8, 8.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	1.19 (1.93)	2.53 (1.00)
Median	1.24	2.29
25th, 75th Percentile	0.07, 3.32	1.65, 3.34
Min, Max	-2.1, 3.5	1.6, 4.3
LS mean change from baseline (95% CI)	1.11 (0.59, 1.62)	2.61 (2.09, 3.13)
Difference in LS mean change from baseline (95% CI) ^a		1.51 (0.76, 2.25)
P-value ^b		0.0014

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=16)	Vosoritide (N=15)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		2.54 (0.92, 4.11)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	3.82 (1.86)	5.00 (2.17)
Median	3.82	5.59
25th, 75th Percentile	3.02, 4.37	4.15, 6.31
Min, Max	0.3, 7.1	0.6, 7.3
Week 52		
n	9	8
Mean (SD)	5.12 (0.80)	5.80 (2.07)
Median	5.41	6.03

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.1
 Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	4.38, 5.85	4.05, 7.59
Min, Max	4.0, 6.2	2.9, 8.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.1
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	1.30 (2.28)	0.80 (3.02)
Median	1.35	0.40
25th, 75th Percentile	0.29, 1.59	-1.42, 2.54
Min, Max	-2.8, 5.8	-3.0, 6.4
LS mean change from baseline (95% CI)	0.56 (-0.70, 1.83)	1.63 (0.27, 2.98)
Difference in LS mean change from baseline (95% CI) ^a		1.06 (-0.90, 3.02)
P-value ^b		0.2605

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.1
 Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex	Placebo (N=16)	Vosoritide (N=15)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		0.65 (-0.47, 1.74)
P-value for interaction term, treatment * [Sex]		0.6994

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	3.92 (1.63)	4.40 (1.86)
Median	4.00	5.30
25th, 75th Percentile	3.02, 4.46	3.40, 5.63
Min, Max	0.3, 7.1	0.6, 5.9
Week 52		
n	13	8
Mean (SD)	5.49 (0.95)	6.67 (1.95)
Median	5.57	7.01

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	4.52, 6.14	5.75, 8.10
Min, Max	4.0, 7.3	2.9, 8.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.2
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Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	1.57 (2.05)	2.27 (2.91)
Median	1.35	2.72
25th, 75th Percentile	0.89, 2.79	0.86, 3.82
Min, Max	-2.8, 5.8	-3.0, 6.4
LS mean change from baseline (95% CI)	1.36 (0.65, 2.08)	2.60 (1.69, 3.52)
Difference in LS mean change from baseline (95% CI) ^a		1.24 (0.07, 2.41)
P-value ^b		0.0390

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		1.03 (0.05, 1.97)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	5.44 (2.24)	5.13 (1.47)
Median	5.16	4.85
25th, 75th Percentile	3.34, 7.80	4.20, 6.68
Min, Max	3.3, 7.8	3.0, 7.3
Week 52		
n	3	7
Mean (SD)	5.30 (0.47)	5.98 (1.42)
Median	5.45	6.09

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	4.77, 5.67	5.04, 7.14
Min, Max	4.8, 5.7	3.4, 7.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	-0.14 (1.82)	0.85 (1.54)
Median	0.29	1.61
25th, 75th Percentile	-2.13, 1.43	0.34, 1.84
Min, Max	-2.1, 1.4	-2.2, 2.3
LS mean change from baseline (95% CI)	1.89 (-4.22, 7.99)	-0.02 (-2.79, 2.76)
Difference in LS mean change from baseline (95% CI) ^a		-1.91 (-10.50, 6.69)
P-value ^b		0.5715

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and ethnicity interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

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Table 14.2.3.2.102.2
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity	Placebo (N=16)	Vosoritide (N=15)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		-1.63 (-6.84, 3.77)
P-value for interaction term,treatment * [Ethnicity]		0.8812

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	5.53 (1.60)	5.52 (1.07)
Median	4.98	5.37
25th, 75th Percentile	4.42, 6.65	4.64, 6.31
Min, Max	4.4, 7.8	4.2, 7.3
Week 52		
n	4	8
Mean (SD)	5.62 (0.23)	5.76 (1.51)
Median	5.62	5.95

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	5.46, 5.79	4.89, 7.14
Min, Max	5.3, 5.9	2.9, 7.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	0.09 (1.60)	0.25 (2.01)
Median	0.48	1.03
25th, 75th Percentile	-1.03, 1.21	-1.42, 1.75
Min, Max	-2.1, 1.5	-3.0, 2.3
LS mean change from baseline (95% CI)	-0.13 (-1.67, 1.41)	0.35 (-0.72, 1.43)
Difference in LS mean change from baseline (95% CI) ^a		0.48 (-1.42, 2.39)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.5684
SMD (95% CI) ^c		0.38 (-0.89, 1.63)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	3.76 (1.67)	3.85 (1.87)
Median	3.72	4.10
25th, 75th Percentile	2.83, 4.70	2.71, 5.41
Min, Max	0.3, 7.1	0.6, 5.8
Week 52		
n	12	7
Mean (SD)	5.40 (1.00)	7.01 (1.77)
Median	5.43	7.01

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	4.45, 6.15	6.76, 8.15
Min, Max	4.0, 7.3	3.4, 8.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.3.2.102.3
Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	1.64 (2.12)	3.16 (1.86)
Median	1.39	2.78
25th, 75th Percentile	0.59, 3.05	2.31, 4.30
Min, Max	-2.8, 5.8	0.3, 6.4
LS mean change from baseline (95% CI)	1.64 (0.82, 2.47)	3.15 (2.07, 4.23)
Difference in LS mean change from baseline (95% CI) ^a		1.51 (0.14, 2.87)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.3.2.102.3

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.0326

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

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Table 14.2.3.2.102.3

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		1.13 (0.09, 2.14)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.2855

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, and treatment and age stratum interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.102.3

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
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^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
Baseline		
n	11	6
Mean (SD)	3.32 (1.19)	3.18 (1.43)
Median	3.63	3.56
25th, 75th Percentile	2.63, 4.27	2.71, 4.20
Min, Max	0.3, 4.5	0.6, 4.4
Week 52		
n	11	6
Mean (SD)	5.44 (0.97)	6.01 (1.38)
Median	5.41	6.42

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	4.52, 6.14	5.81, 7.01
Min, Max	4.0, 7.3	3.4, 7.0

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	2.12 (1.61)	2.83 (2.19)
Median	1.53	2.16
25th, 75th Percentile	0.89, 3.32	1.61, 4.30
Min, Max	0.2, 5.8	0.3, 6.4
LS mean change from baseline (95% CI)	2.33 (1.58, 3.09)	2.44 (1.39, 3.49)
Difference in LS mean change from baseline (95% CI) ^a		0.10 (-1.24, 1.45)
P-value ^b		0.8668

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

Note one subject had no height assessment within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.09 (-0.99, 1.18)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	6.15 (1.24)	5.78 (0.77)
Median	5.51	5.42
25th, 75th Percentile	5.16, 7.14	5.33, 5.94
Min, Max	5.1, 7.8	4.9, 7.3
Week 52		
n	5	9
Mean (SD)	5.49 (0.72)	6.57 (1.93)
Median	5.57	7.14

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, and treatment and AGV interaction.

Z-Scores were derived using age-sex specific reference data (means and SDs) for average stature children per the Centers for Disease Control and Prevention.

Subjects aged < 24 months, body length takes precedence over standing height. Subjects aged < 24 months at baseline and ≥ 24 months at Week 52, body length takes precedence.

If there was no height assessment within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute missing height/body length.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	5.45, 5.67	5.04, 8.05
Min, Max	4.4, 6.4	2.9, 8.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	-0.66 (1.71)	0.79 (2.29)
Median	0.07	1.84
25th, 75th Percentile	-2.13, 0.29	-0.60, 2.31
Min, Max	-2.8, 1.2	-3.0, 3.3
LS mean change from baseline (95% CI)	-1.29 (-2.62, 0.05)	1.14 (0.19, 2.08)
Difference in LS mean change from baseline (95% CI) ^a		2.42 (0.66, 4.18)
P-value ^b		0.0131

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.2.102.4

Analysis of Covariance of Cumulative Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		2.13 (0.42, 3.75)
P-value for interaction term, treatment * [Baseline AGV]		0.1553

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	16
Mean (SD)	2.45 (0.31)	2.51 (0.32)
Median	2.43	2.51
25th, 75th Percentile	2.27, 2.57	2.26, 2.64
Min, Max	2.0, 3.1	2.0, 3.1

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.001_mod_sub_sex_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	13	16
Mean (SD)	2.36 (0.26)	2.36 (0.28)
Median	2.34	2.39
25th, 75th Percentile	2.23, 2.45	2.10, 2.54
Min, Max	1.9, 2.9	1.9, 2.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	16
Mean (SD)	-0.09 (0.14)	-0.15 (0.22)
Median	-0.12	-0.15
25th, 75th Percentile	-0.18, -0.06	-0.26, -0.07
Min, Max	-0.3, 0.2	-0.7, 0.3
LS mean change from baseline (95% CI)	-0.09 (-0.18, 0.01)	-0.15 (-0.24, -0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.20, 0.06)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.2861
SMD (95% CI) ^c		-0.43 (-1.19, 0.35)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.001_mod_sub_sex_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Female		
Baseline		
n	19	15
Mean (SD)	2.56 (0.39)	2.68 (0.48)
Median	2.63	2.48
25th, 75th Percentile	2.22, 2.91	2.42, 3.04
Min, Max	1.9, 3.2	2.2, 4.0

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	19	15
Mean (SD)	2.41 (0.33)	2.37 (0.27)
Median	2.40	2.42
25th, 75th Percentile	2.18, 2.69	2.21, 2.55
Min, Max	1.7, 2.9	1.7, 2.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	19	15
Mean (SD)	-0.14 (0.25)	-0.31 (0.36)
Median	-0.12	-0.13
25th, 75th Percentile	-0.24, 0.03	-0.68, -0.08
Min, Max	-0.8, 0.2	-1.1, 0.2
LS mean change from baseline (95% CI)	-0.17 (-0.26, -0.07)	-0.28 (-0.39, -0.17)
Difference in LS mean change from baseline (95% CI) ^a		-0.12 (-0.27, 0.04)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.1328
SMD (95% CI) ^c		-0.60 (-1.38, 0.18)
P-value for interaction term, treatment * [Sex]		0.6026

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.001_mod_sub_sex_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	25	20
Mean (SD)	2.50 (0.35)	2.65 (0.45)
Median	2.50	2.51
25th, 75th Percentile	2.30, 2.75	2.36, 2.87
Min, Max	1.9, 3.2	2.1, 4.0

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.002_mod_sub_eth_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	25	20
Mean (SD)	2.37 (0.32)	2.36 (0.27)
Median	2.36	2.40
25th, 75th Percentile	2.18, 2.63	2.17, 2.47
Min, Max	1.7, 2.9	1.7, 2.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	25	20
Mean (SD)	-0.13 (0.21)	-0.29 (0.33)
Median	-0.12	-0.13
25th, 75th Percentile	-0.20, 0.00	-0.54, -0.08
Min, Max	-0.8, 0.2	-1.1, 0.2
LS mean change from baseline (95% CI)	-0.16 (-0.24, -0.09)	-0.25 (-0.33, -0.16)
Difference in LS mean change from baseline (95% CI) ^a		-0.09 (-0.20, 0.03)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.1429
SMD (95% CI) ^c		-0.47 (-1.09, 0.16)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Non-White		
Baseline		
n	7	11
Mean (SD)	2.57 (0.41)	2.50 (0.33)
Median	2.43	2.42
25th, 75th Percentile	2.22, 3.01	2.23, 2.65
Min, Max	2.1, 3.0	2.0, 3.1

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	7	11
Mean (SD)	2.45 (0.27)	2.38 (0.29)
Median	2.32	2.39
25th, 75th Percentile	2.26, 2.77	2.11, 2.56
Min, Max	2.2, 2.9	1.9, 2.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.002_mod_sub_eth_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	11
Mean (SD)	-0.11 (0.23)	-0.12 (0.22)
Median	-0.09	-0.19
25th, 75th Percentile	-0.27, 0.04	-0.21, 0.07
Min, Max	-0.5, 0.2	-0.5, 0.3
LS mean change from baseline (95% CI)	-0.10 (-0.29, 0.09)	-0.12 (-0.27, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.28, 0.23)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.8525
SMD (95% CI) ^c		-0.10 (-1.16, 0.95)
P-value for interaction term, treatment * [Ethnicity]		0.6138

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	2.30 (0.08)	2.39 (0.20)
Median	2.31	2.45
25th, 75th Percentile	2.24, 2.37	2.23, 2.53
Min, Max	2.2, 2.4	2.0, 2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	4	8
Mean (SD)	2.25 (0.16)	2.31 (0.21)
Median	2.20	2.41
25th, 75th Percentile	2.15, 2.35	2.16, 2.44
Min, Max	2.1, 2.5	1.9, 2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	-0.05 (0.13)	-0.08 (0.21)
Median	-0.08	-0.13
25th, 75th Percentile	-0.13, 0.03	-0.21, 0.01
Min, Max	-0.2, 0.1	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.07 (-0.35, 0.20)	-0.07 (-0.26, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.34, 0.35)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.3
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.9737
SMD (95% CI) ^c		0.02 (-1.25, 1.29)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	2.24 (0.22)	2.30 (0.13)
Median	2.26	2.35
25th, 75th Percentile	2.06, 2.44	2.19, 2.42
Min, Max	1.9, 2.5	2.1, 2.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	12	7
Mean (SD)	2.14 (0.22)	2.09 (0.20)
Median	2.21	2.08
25th, 75th Percentile	2.01, 2.28	2.02, 2.30
Min, Max	1.7, 2.4	1.7, 2.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	-0.10 (0.16)	-0.21 (0.23)
Median	-0.10	-0.11
25th, 75th Percentile	-0.18, 0.02	-0.31, -0.08
Min, Max	-0.5, 0.2	-0.7, -0.1
LS mean change from baseline (95% CI)	-0.10 (-0.22, 0.02)	-0.20 (-0.35, -0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.10 (-0.30, 0.10)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.3167
SMD (95% CI) ^c		-0.50 (-1.46, 0.47)
P-value for interaction term, treatment * [[Cohort 1]Age stratum]		0.5081

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	7
Mean (SD)	2.33 (0.31)	2.30 (0.13)
Median	2.38	2.35
25th, 75th Percentile	2.12, 2.47	2.23, 2.42
Min, Max	1.9, 3.1	2.0, 2.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	12	7
Mean (SD)	2.22 (0.30)	2.15 (0.22)
Median	2.23	2.11
25th, 75th Percentile	2.12, 2.29	2.04, 2.34
Min, Max	1.7, 2.9	1.7, 2.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	-0.11 (0.15)	-0.16 (0.31)
Median	-0.12	-0.12
25th, 75th Percentile	-0.19, -0.03	-0.31, -0.06
Min, Max	-0.5, 0.2	-0.7, 0.3
LS mean change from baseline (95% CI)	-0.09 (-0.24, 0.06)	-0.20 (-0.40, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.11 (-0.37, 0.15)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.004_mod_sub_agv_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.3888
SMD (95% CI) ^c		-0.47 (-1.49, 0.58)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	20	24
Mean (SD)	2.63 (0.35)	2.68 (0.43)
Median	2.64	2.56
25th, 75th Percentile	2.32, 2.92	2.44, 3.02
Min, Max	2.0, 3.2	2.1, 4.0

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	20	24
Mean (SD)	2.50 (0.26)	2.43 (0.25)
Median	2.49	2.44
25th, 75th Percentile	2.33, 2.73	2.28, 2.60
Min, Max	1.9, 2.9	1.9, 2.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	20	24
Mean (SD)	-0.13 (0.24)	-0.25 (0.31)
Median	-0.12	-0.15
25th, 75th Percentile	-0.24, 0.04	-0.40, -0.08
Min, Max	-0.8, 0.2	-1.1, 0.2
LS mean change from baseline (95% CI)	-0.16 (-0.24, -0.08)	-0.22 (-0.30, -0.15)
Difference in LS mean change from baseline (95% CI) ^a		-0.06 (-0.18, 0.05)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.2742
SMD (95% CI) ^c		-0.36 (-0.98, 0.28)
P-value for interaction term, treatment * [Baseline AGV]		0.8534

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.5
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	18	13
Mean (SD)	2.43 (0.34)	2.41 (0.27)
Median	2.40	2.42
25th, 75th Percentile	2.22, 2.58	2.23, 2.48
Min, Max	1.9, 3.1	2.0, 3.1

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and height z-score interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.5
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	18	13
Mean (SD)	2.32 (0.30)	2.28 (0.28)
Median	2.28	2.30
25th, 75th Percentile	2.18, 2.48	2.11, 2.44
Min, Max	1.7, 2.9	1.7, 2.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and height z-score interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.5
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	13
Mean (SD)	-0.11 (0.15)	-0.13 (0.25)
Median	-0.13	-0.12
25th, 75th Percentile	-0.21, 0.00	-0.21, -0.06
Min, Max	-0.5, 0.2	-0.7, 0.3
LS mean change from baseline (95% CI)	-0.10 (-0.20, 0.00)	-0.15 (-0.27, -0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.21, 0.11)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and height z-score interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.5
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.4966
SMD (95% CI) ^c		-0.28 (-1.08, 0.53)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and height z-score interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.101.5
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	18
Mean (SD)	2.62 (0.37)	2.72 (0.45)
Median	2.64	2.56
25th, 75th Percentile	2.39, 2.93	2.45, 3.04
Min, Max	2.0, 3.2	2.2, 4.0

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and height z-score interaction.

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If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.101.5
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Week 52		
n	14	18
Mean (SD)	2.49 (0.29)	2.43 (0.25)
Median	2.46	2.43
25th, 75th Percentile	2.32, 2.78	2.33, 2.56
Min, Max	1.9, 2.9	1.9, 2.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

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Table 14.2.5.2.101.5
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	18
Mean (SD)	-0.14 (0.27)	-0.30 (0.33)
Median	-0.10	-0.18
25th, 75th Percentile	-0.18, 0.03	-0.48, -0.10
Min, Max	-0.8, 0.2	-1.1, 0.2
LS mean change from baseline (95% CI)	-0.21 (-0.32, -0.10)	-0.24 (-0.33, -0.15)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.18, 0.12)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

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Table 14.2.5.2.101.5
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper to Lower Body Segment Ratio	Placebo (N=32)	Vosoritide (N=32)
P-value ^b		0.6918
SMD (95% CI) ^c		-0.17 (-0.97, 0.65)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.8299

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and height z-score interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.101.005_mod_sub_haz_bod_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	2.31 (0.17)	2.28 (0.20)
Median	2.34	2.23
25th, 75th Percentile	2.21, 2.44	2.09, 2.36
Min, Max	2.0, 2.5	2.0, 2.7
Week 52		
n	7	7
Mean (SD)	2.23 (0.20)	2.17 (0.20)
Median	2.23	2.11

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.13, 2.45	2.02, 2.39
Min, Max	1.9, 2.5	1.9, 2.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	-0.08 (0.11)	-0.11 (0.23)
Median	-0.09	-0.12
25th, 75th Percentile	-0.16, -0.06	-0.31, -0.06
Min, Max	-0.2, 0.1	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.06 (-0.22, 0.10)	-0.13 (-0.29, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.31, 0.16)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.4966
SMD (95% CI) ^c		-0.40 (-1.51, 0.73)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	2.21 (0.20)	2.40 (0.13)
Median	2.22	2.43
25th, 75th Percentile	2.11, 2.36	2.32, 2.49
Min, Max	1.9, 2.5	2.2, 2.6
Week 52		
n	9	8
Mean (SD)	2.12 (0.21)	2.24 (0.26)
Median	2.18	2.27

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.11, 2.26	2.09, 2.43
Min, Max	1.7, 2.3	1.7, 2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	-0.09 (0.18)	-0.16 (0.22)
Median	-0.08	-0.12
25th, 75th Percentile	-0.18, 0.03	-0.17, -0.07
Min, Max	-0.5, 0.2	-0.7, 0.1
LS mean change from baseline (95% CI)	-0.09 (-0.25, 0.07)	-0.17 (-0.34, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.08 (-0.34, 0.18)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.5099

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.40 (-1.56, 0.78)
P-value for interaction term, treatment * [Sex]		0.8809

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and sex interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.001_mod_sub_sex_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	2.24 (0.21)	2.34 (0.16)
Median	2.30	2.36
25th, 75th Percentile	2.11, 2.39	2.21, 2.46
Min, Max	1.9, 2.5	2.1, 2.6
Week 52		
n	13	8
Mean (SD)	2.15 (0.23)	2.15 (0.23)
Median	2.18	2.09

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.002_mod_sub_eth_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.11, 2.28	2.03, 2.36
Min, Max	1.7, 2.5	1.7, 2.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	-0.09 (0.16)	-0.19 (0.21)
Median	-0.08	-0.12
25th, 75th Percentile	-0.18, 0.00	-0.22, -0.07
Min, Max	-0.5, 0.2	-0.7, -0.1
LS mean change from baseline (95% CI)	-0.09 (-0.20, 0.02)	-0.19 (-0.33, -0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.10 (-0.28, 0.09)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.002_mod_sub_eth_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.2693
SMD (95% CI) ^c		-0.55 (-1.51, 0.42)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.002_mod_sub_eth_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	2.31 (0.11)	2.35 (0.20)
Median	2.27	2.42
25th, 75th Percentile	2.22, 2.43	2.23, 2.48
Min, Max	2.2, 2.4	2.0, 2.7
Week 52		
n	3	7
Mean (SD)	2.24 (0.05)	2.28 (0.22)
Median	2.26	2.34

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.18, 2.28	2.11, 2.44
Min, Max	2.2, 2.3	1.9, 2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	-0.07 (0.10)	-0.08 (0.22)
Median	-0.09	-0.12
25th, 75th Percentile	-0.16, 0.04	-0.21, 0.07
Min, Max	-0.2, 0.0	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.60 (-3.28, 2.08)	0.15 (-1.02, 1.32)
Difference in LS mean change from baseline (95% CI) ^a		0.75 (-3.05, 4.56)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.5739

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.2
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		2.98 (-6.80, 12.32)
P-value for interaction term, treatment * [Ethnicity]		0.6888

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and ethnicity interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	2.30 (0.08)	2.39 (0.20)
Median	2.31	2.45
25th, 75th Percentile	2.24, 2.37	2.23, 2.53
Min, Max	2.2, 2.4	2.0, 2.7
Week 52		
n	4	8
Mean (SD)	2.25 (0.16)	2.31 (0.21)
Median	2.20	2.41

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.15, 2.35	2.16, 2.44
Min, Max	2.1, 2.5	1.9, 2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	-0.05 (0.13)	-0.08 (0.21)
Median	-0.08	-0.13
25th, 75th Percentile	-0.13, 0.03	-0.21, 0.01
Min, Max	-0.2, 0.1	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.07 (-0.35, 0.20)	-0.07 (-0.26, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.34, 0.35)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.9737
SMD (95% CI) ^c		0.02 (-1.25, 1.29)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	2.24 (0.22)	2.30 (0.13)
Median	2.26	2.35
25th, 75th Percentile	2.06, 2.44	2.19, 2.42
Min, Max	1.9, 2.5	2.1, 2.4
Week 52		
n	12	7
Mean (SD)	2.14 (0.22)	2.09 (0.20)
Median	2.21	2.08

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.01, 2.28	2.02, 2.30
Min, Max	1.7, 2.4	1.7, 2.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	-0.10 (0.16)	-0.21 (0.23)
Median	-0.10	-0.11
25th, 75th Percentile	-0.18, 0.02	-0.31, -0.08
Min, Max	-0.5, 0.2	-0.7, -0.1
LS mean change from baseline (95% CI)	-0.10 (-0.22, 0.02)	-0.20 (-0.35, -0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.10 (-0.30, 0.10)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.3167

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.003_mod_sub_strat_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.50 (-1.46, 0.47)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.5081

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.003_mod_sub_strat_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.3

Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
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^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and age stratum interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.003_mod_sub_strat_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
Baseline		
n	11	6
Mean (SD)	2.27 (0.22)	2.31 (0.15)
Median	2.36	2.36
25th, 75th Percentile	2.11, 2.44	2.23, 2.42
Min, Max	1.9, 2.5	2.0, 2.4
Week 52		
n	11	6
Mean (SD)	2.16 (0.21)	2.16 (0.24)
Median	2.22	2.20

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.004_mod_sub_agv_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.11, 2.28	2.04, 2.34
Min, Max	1.7, 2.4	1.7, 2.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.004_mod_sub_agv_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	-0.11 (0.16)	-0.15 (0.34)
Median	-0.08	-0.10
25th, 75th Percentile	-0.20, 0.00	-0.31, -0.06
Min, Max	-0.5, 0.2	-0.7, 0.3
LS mean change from baseline (95% CI)	-0.10 (-0.26, 0.06)	-0.17 (-0.39, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.06 (-0.35, 0.22)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.6202
SMD (95% CI) ^c		-0.28 (-1.37, 0.81)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	2.23 (0.14)	2.37 (0.19)
Median	2.27	2.42
25th, 75th Percentile	2.22, 2.30	2.23, 2.50
Min, Max	2.0, 2.3	2.1, 2.7
Week 52		
n	5	9
Mean (SD)	2.20 (0.21)	2.24 (0.23)
Median	2.18	2.21

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged < 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	2.18, 2.26	2.08, 2.44
Min, Max	1.9, 2.5	1.9, 2.5

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	-0.03 (0.11)	-0.13 (0.11)
Median	-0.09	-0.12
25th, 75th Percentile	-0.12, 0.04	-0.21, -0.08
Min, Max	-0.1, 0.1	-0.3, 0.1
LS mean change from baseline (95% CI)	-0.01 (-0.18, 0.15)	-0.14 (-0.26, -0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.13 (-0.35, 0.09)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged ≥ 24 months at baseline and ≥ 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
P-value ^b		0.2116

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.002.102.004_mod_sub_agv_bod_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.2.102.4
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.93 (-2.32, 0.51)
P-value for interaction term,treatment * [Baseline AGV]		0.8834

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper to lower body segment ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper to lower body segment ratio, and treatment and AGV interaction.

Subjects aged < 24 months, body length and crown to rump length take precedence over standing height and sitting height. Subjects aged >= 24 months at baseline and >= 24 months at Week 52, body length and crown to rump length take precedence.

If there was no upper to lower body segment ratio within the Week 52 analysis visit window, but there are assessments before and after Week 52, a linear interpolation using the measurements closest to the before and after Week 52 will be used to impute upper to lower body segment ratio.

Note one subject had no upper to lower body segment ratio within the Week 52 analysis visit nor after Week 52 and was excluded from this analysis.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	14
Mean (SD)	1.13 (0.10)	1.07 (0.05)
Median	1.09	1.06
25th, 75th Percentile	1.05, 1.20	1.03, 1.11
Min, Max	1.0, 1.3	1.0, 1.2
Week 52		
n	13	14
Mean (SD)	1.10 (0.12)	1.04 (0.10)
Median	1.05	1.03
25th, 75th Percentile	1.04, 1.11	0.94, 1.10
Min, Max	1.0, 1.4	0.9, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.001_mod_sub_sex_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	14
Mean (SD)	-0.03 (0.12)	-0.03 (0.09)
Median	-0.05	-0.03
25th, 75th Percentile	-0.08, 0.02	-0.10, 0.00
Min, Max	-0.2, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.01 (-0.08, 0.05)	-0.04 (-0.11, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.13, 0.07)
P-value ^b		0.5302

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.001_mod_sub_sex_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.28 (-1.13, 0.58)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.001_mod_sub_sex_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Female		
Baseline		
n	18	15
Mean (SD)	1.05 (0.08)	1.07 (0.09)
Median	1.06	1.08
25th, 75th Percentile	1.00, 1.09	1.01, 1.13
Min, Max	0.9, 1.2	0.9, 1.2
Week 52		
n	18	15
Mean (SD)	1.08 (0.11)	1.09 (0.13)
Median	1.08	1.08
25th, 75th Percentile	1.03, 1.12	1.00, 1.16
Min, Max	0.9, 1.4	0.9, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.001_mod_sub_sex_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	15
Mean (SD)	0.04 (0.15)	0.02 (0.15)
Median	0.02	0.00
25th, 75th Percentile	-0.07, 0.10	-0.10, 0.15
Min, Max	-0.2, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	0.03 (-0.03, 0.09)	0.03 (-0.04, 0.10)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.10, 0.10)
P-value ^b		0.9585

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.001_mod_sub_sex_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.02 (-0.79, 0.75)
P-value for interaction term, treatment * [Sex]		0.5051

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.001_mod_sub_sex_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	24	19
Mean (SD)	1.08 (0.09)	1.07 (0.08)
Median	1.07	1.04
25th, 75th Percentile	1.02, 1.12	1.01, 1.11
Min, Max	0.9, 1.3	0.9, 1.2
Week 52		
n	24	19
Mean (SD)	1.09 (0.10)	1.02 (0.08)
Median	1.07	1.01
25th, 75th Percentile	1.04, 1.11	0.94, 1.09
Min, Max	0.9, 1.4	0.9, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.002_mod_sub_eth_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	24	19
Mean (SD)	0.01 (0.14)	-0.04 (0.11)
Median	-0.02	-0.07
25th, 75th Percentile	-0.09, 0.07	-0.11, 0.06
Min, Max	-0.2, 0.4	-0.2, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.05)	-0.05 (-0.10, -0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.06 (-0.12, 0.00)
P-value ^b		0.0498

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.002_mod_sub_eth_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.64 (-1.27, 0.00)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.002_mod_sub_eth_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Non-White		
Baseline		
n	7	10
Mean (SD)	1.08 (0.13)	1.09 (0.05)
Median	1.09	1.09
25th, 75th Percentile	0.99, 1.14	1.05, 1.13
Min, Max	0.9, 1.3	1.0, 1.2
Week 52		
n	7	10
Mean (SD)	1.11 (0.14)	1.16 (0.14)
Median	1.06	1.15
25th, 75th Percentile	1.01, 1.15	1.05, 1.23
Min, Max	1.0, 1.4	1.0, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.002_mod_sub_eth_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	10
Mean (SD)	0.03 (0.14)	0.07 (0.13)
Median	0.02	0.01
25th, 75th Percentile	-0.08, 0.13	-0.03, 0.18
Min, Max	-0.1, 0.3	0.0, 0.3
LS mean change from baseline (95% CI)	0.02 (-0.09, 0.13)	0.08 (-0.01, 0.16)
Difference in LS mean change from baseline (95% CI) ^a		0.06 (-0.09, 0.21)
P-value ^b		0.4052

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.002_mod_sub_eth_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity	Placebo (N=32)	Vosoritide (N=32)
Upper Arm Length to Lower Arm (Forearm) Length Ratio		
SMD (95% CI) ^c		0.50 (-0.66, 1.64)
P-value for interaction term,treatment * [Ethnicity]		0.1767

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.002_mod_sub_eth_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.3
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	1.11 (0.12)	1.09 (0.07)
Median	1.07	1.10
25th, 75th Percentile	1.04, 1.18	1.04, 1.14
Min, Max	1.0, 1.3	1.0, 1.2
Week 52		
n	4	8
Mean (SD)	1.16 (0.17)	1.11 (0.08)
Median	1.10	1.09
25th, 75th Percentile	1.04, 1.29	1.06, 1.15
Min, Max	1.0, 1.4	1.0, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.003_mod_sub_strat_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.3
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	0.06 (0.07)	0.02 (0.10)
Median	0.06	-0.01
25th, 75th Percentile	-0.01, 0.12	-0.04, 0.08
Min, Max	0.0, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.06 (-0.03, 0.16)	0.02 (-0.05, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.16, 0.06)
P-value ^b		0.3265

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.003_mod_sub_strat_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.68 (-1.95, 0.65)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.003_mod_sub_strat_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.3
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	1.07 (0.10)	1.11 (0.06)
Median	1.07	1.11
25th, 75th Percentile	1.00, 1.10	1.08, 1.12
Min, Max	0.9, 1.2	1.0, 1.2
Week 52		
n	12	7
Mean (SD)	1.11 (0.13)	1.08 (0.17)
Median	1.09	1.01
25th, 75th Percentile	1.03, 1.13	1.00, 1.10
Min, Max	1.0, 1.4	0.9, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.003_mod_sub_strat_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.3
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	0.05 (0.18)	-0.04 (0.17)
Median	0.01	-0.08
25th, 75th Percentile	-0.09, 0.18	-0.16, 0.00
Min, Max	-0.1, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	0.03 (-0.07, 0.13)	0.00 (-0.13, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.20, 0.13)
P-value ^b		0.6630

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.003_mod_sub_strat_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.3
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.22 (-1.20, 0.76)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.9959

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.003_mod_sub_strat_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	6
Mean (SD)	1.08 (0.09)	1.11 (0.08)
Median	1.07	1.11
25th, 75th Percentile	1.02, 1.10	1.04, 1.12
Min, Max	1.0, 1.3	1.0, 1.2
Week 52		
n	12	6
Mean (SD)	1.11 (0.13)	1.12 (0.16)
Median	1.07	1.08
25th, 75th Percentile	1.03, 1.14	1.01, 1.10
Min, Max	1.0, 1.4	1.0, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.004_mod_sub_agv_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	6
Mean (SD)	0.03 (0.17)	0.01 (0.16)
Median	0.02	-0.02
25th, 75th Percentile	-0.09, 0.10	-0.04, 0.00
Min, Max	-0.2, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	-0.01 (-0.10, 0.08)	0.10 (-0.04, 0.23)
Difference in LS mean change from baseline (95% CI) ^a		0.11 (-0.06, 0.28)
P-value ^b		0.1889

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.004_mod_sub_agv_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.87 (-0.41, 2.11)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.004_mod_sub_agv_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	19	23
Mean (SD)	1.08 (0.10)	1.07 (0.07)
Median	1.08	1.06
25th, 75th Percentile	1.02, 1.14	1.02, 1.12
Min, Max	0.9, 1.3	0.9, 1.2
Week 52		
n	19	23
Mean (SD)	1.08 (0.10)	1.05 (0.11)
Median	1.06	1.05
25th, 75th Percentile	1.03, 1.11	0.97, 1.12
Min, Max	0.9, 1.4	0.9, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.004_mod_sub_agv_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	19	23
Mean (SD)	0.00 (0.12)	-0.01 (0.12)
Median	-0.03	-0.03
25th, 75th Percentile	-0.08, 0.05	-0.10, 0.07
Min, Max	-0.2, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.03, 0.06)	-0.03 (-0.07, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.04 (-0.11, 0.02)
P-value ^b		0.1888

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.004_mod_sub_agv_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.43 (-1.07, 0.21)
P-value for interaction term, treatment * [Baseline AGV]		0.5483

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.004_mod_sub_agv_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.5
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	17	13
Mean (SD)	1.09 (0.11)	1.11 (0.05)
Median	1.09	1.11
25th, 75th Percentile	1.05, 1.14	1.10, 1.13
Min, Max	0.9, 1.3	1.0, 1.2
Week 52		
n	17	13
Mean (SD)	1.11 (0.14)	1.10 (0.14)
Median	1.07	1.09
25th, 75th Percentile	1.04, 1.12	1.01, 1.18
Min, Max	1.0, 1.4	0.9, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.005_mod_sub_haz_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.5
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	17	13
Mean (SD)	0.03 (0.16)	-0.01 (0.15)
Median	-0.01	-0.03
25th, 75th Percentile	-0.07, 0.10	-0.11, 0.04
Min, Max	-0.2, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	0.01 (-0.06, 0.09)	0.00 (-0.08, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.13, 0.11)
P-value ^b		0.8484

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.005_mod_sub_haz_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.5
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.08 (-0.89, 0.73)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.005_mod_sub_haz_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.5
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	16
Mean (SD)	1.07 (0.08)	1.04 (0.07)
Median	1.06	1.03
25th, 75th Percentile	1.02, 1.11	1.01, 1.06
Min, Max	0.9, 1.2	0.9, 1.2
Week 52		
n	14	16
Mean (SD)	1.06 (0.07)	1.04 (0.09)
Median	1.07	1.03
25th, 75th Percentile	1.03, 1.11	0.98, 1.10
Min, Max	0.9, 1.2	0.9, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.005_mod_sub_haz_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.5
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	16
Mean (SD)	-0.01 (0.10)	0.00 (0.11)
Median	-0.01	-0.02
25th, 75th Percentile	-0.08, 0.05	-0.09, 0.08
Min, Max	-0.2, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.04, 0.05)	-0.01 (-0.05, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.08, 0.05)
P-value ^b		0.6834

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.005_mod_sub_haz_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.101.5
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.16 (-0.93, 0.61)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.9510

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.101.005_mod_sub_haz_armrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.1

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	1.11 (0.11)	1.09 (0.05)
Median	1.08	1.10
25th, 75th Percentile	1.02, 1.24	1.04, 1.12
Min, Max	1.0, 1.3	1.0, 1.2
Week 52		
n	7	7
Mean (SD)	1.14 (0.16)	1.04 (0.07)
Median	1.05	1.05
25th, 75th Percentile	1.04, 1.31	1.01, 1.10
Min, Max	1.0, 1.4	0.9, 1.1

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.3.102.1

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	0.03 (0.13)	-0.05 (0.06)
Median	0.02	-0.03
25th, 75th Percentile	-0.05, 0.13	-0.04, -0.01
Min, Max	-0.1, 0.3	-0.2, 0.0
LS mean change from baseline (95% CI)	0.03 (-0.07, 0.12)	-0.04 (-0.14, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.21, 0.07)
P-value ^b		0.2652

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.001_mod_sub_sex_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.1

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.67 (-1.80, 0.49)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.001_mod_sub_sex_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.1

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	1.05 (0.10)	1.12 (0.07)
Median	1.06	1.11
25th, 75th Percentile	1.02, 1.09	1.08, 1.15
Min, Max	0.9, 1.2	1.0, 1.2
Week 52		
n	9	8
Mean (SD)	1.12 (0.13)	1.14 (0.15)
Median	1.10	1.09
25th, 75th Percentile	1.04, 1.15	1.04, 1.23
Min, Max	1.0, 1.4	1.0, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.3.102.1

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	0.06 (0.18)	0.03 (0.17)
Median	0.02	-0.01
25th, 75th Percentile	-0.07, 0.10	-0.10, 0.15
Min, Max	-0.1, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	0.04 (-0.09, 0.17)	0.06 (-0.08, 0.20)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.20, 0.23)
P-value ^b		0.8816

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.001_mod_sub_sex_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.1

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.10 (-1.15, 1.34)
P-value for interaction term, treatment * [Sex]		0.5800

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.001_mod_sub_sex_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	1.08 (0.08)	1.11 (0.08)
Median	1.08	1.11
25th, 75th Percentile	1.05, 1.09	1.06, 1.15
Min, Max	1.0, 1.2	1.0, 1.2
Week 52		
n	13	8
Mean (SD)	1.11 (0.13)	1.04 (0.06)
Median	1.07	1.04
25th, 75th Percentile	1.04, 1.12	1.00, 1.09
Min, Max	1.0, 1.4	0.9, 1.1

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.002_mod_sub_eth_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	0.03 (0.16)	-0.07 (0.09)
Median	-0.01	-0.09
25th, 75th Percentile	-0.07, 0.10	-0.14, -0.02
Min, Max	-0.1, 0.4	-0.2, 0.1
LS mean change from baseline (95% CI)	0.02 (-0.06, 0.09)	-0.05 (-0.15, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.19, 0.05)
P-value ^b		0.2240

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.002_mod_sub_eth_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.2
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for
 BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.59 (-1.51, 0.35)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.002_mod_sub_eth_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	1.05 (0.21)	1.10 (0.05)
Median	0.99	1.11
25th, 75th Percentile	0.86, 1.28	1.06, 1.13
Min, Max	0.9, 1.3	1.0, 1.2
Week 52		
n	3	7
Mean (SD)	1.19 (0.20)	1.17 (0.15)
Median	1.15	1.12
25th, 75th Percentile	1.01, 1.41	1.05, 1.28
Min, Max	1.0, 1.4	1.0, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.002_mod_sub_eth_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	0.15 (0.13)	0.07 (0.14)
Median	0.13	-0.01
25th, 75th Percentile	0.02, 0.28	-0.03, 0.20
Min, Max	0.0, 0.3	0.0, 0.3
LS mean change from baseline (95% CI)	0.13 (-0.54, 0.79)	0.08 (-0.22, 0.37)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.98, 0.89)
P-value ^b		0.8809

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.002_mod_sub_eth_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.2
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.51 (-6.67, 5.72)
P-value for interaction term, treatment * [Ethnicity]		0.5914

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.002_mod_sub_eth_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	1.11 (0.12)	1.09 (0.07)
Median	1.07	1.10
25th, 75th Percentile	1.04, 1.18	1.04, 1.14
Min, Max	1.0, 1.3	1.0, 1.2
Week 52		
n	4	8
Mean (SD)	1.16 (0.17)	1.11 (0.08)
Median	1.10	1.09
25th, 75th Percentile	1.04, 1.29	1.06, 1.15
Min, Max	1.0, 1.4	1.0, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.3.102.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	0.06 (0.07)	0.02 (0.10)
Median	0.06	-0.01
25th, 75th Percentile	-0.01, 0.12	-0.04, 0.08
Min, Max	0.0, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.06 (-0.03, 0.16)	0.02 (-0.05, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.16, 0.06)
P-value ^b		0.3265

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.003_mod_sub_strat_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.68 (-1.95, 0.65)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.3.102.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	1.07 (0.10)	1.11 (0.06)
Median	1.07	1.11
25th, 75th Percentile	1.00, 1.10	1.08, 1.12
Min, Max	0.9, 1.2	1.0, 1.2
Week 52		
n	12	7
Mean (SD)	1.11 (0.13)	1.08 (0.17)
Median	1.09	1.01
25th, 75th Percentile	1.03, 1.13	1.00, 1.10
Min, Max	1.0, 1.4	0.9, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.3.102.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	0.05 (0.18)	-0.04 (0.17)
Median	0.01	-0.08
25th, 75th Percentile	-0.09, 0.18	-0.16, 0.00
Min, Max	-0.1, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	0.03 (-0.07, 0.13)	0.00 (-0.13, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.20, 0.13)
P-value ^b		0.6630

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.3.102.3

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.22 (-1.20, 0.76)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.9959

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.003_mod_sub_strat_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
Baseline		
n	11	6
Mean (SD)	1.06 (0.07)	1.11 (0.08)
Median	1.06	1.11
25th, 75th Percentile	1.02, 1.09	1.04, 1.12
Min, Max	1.0, 1.2	1.0, 1.2
Week 52		
n	11	6
Mean (SD)	1.11 (0.14)	1.12 (0.16)
Median	1.07	1.08
25th, 75th Percentile	1.02, 1.16	1.01, 1.10
Min, Max	1.0, 1.4	1.0, 1.4

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.004_mod_sub_agv_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	0.05 (0.16)	0.01 (0.16)
Median	0.02	-0.02
25th, 75th Percentile	-0.07, 0.10	-0.04, 0.00
Min, Max	-0.1, 0.4	-0.2, 0.3
LS mean change from baseline (95% CI)	0.00 (-0.09, 0.09)	0.11 (-0.02, 0.24)
Difference in LS mean change from baseline (95% CI) ^a		0.11 (-0.06, 0.28)
P-value ^b		0.1889

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.004_mod_sub_agv_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.4

Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.87 (-0.41, 2.11)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.004_mod_sub_agv_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	1.10 (0.17)	1.10 (0.06)
Median	1.08	1.11
25th, 75th Percentile	1.05, 1.24	1.08, 1.13
Min, Max	0.9, 1.3	1.0, 1.2
Week 52		
n	5	9
Mean (SD)	1.15 (0.15)	1.08 (0.11)
Median	1.11	1.08
25th, 75th Percentile	1.05, 1.15	1.01, 1.12
Min, Max	1.0, 1.4	0.9, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.004_mod_sub_agv_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	0.05 (0.16)	-0.02 (0.12)
Median	-0.01	-0.04
25th, 75th Percentile	-0.03, 0.13	-0.10, 0.05
Min, Max	-0.1, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	0.07 (-0.03, 0.16)	-0.03 (-0.10, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.10 (-0.23, 0.03)
P-value ^b		0.1168

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.004_mod_sub_agv_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.3.102.4
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-1.27 (-2.78, 0.30)
P-value for interaction term, treatment * [Baseline AGV]		0.2321

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper arm length to lower arm length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.003.102.004_mod_sub_agv_armrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	14
Mean (SD)	0.62 (0.11)	0.66 (0.10)
Median	0.64	0.67
25th, 75th Percentile	0.57, 0.67	0.63, 0.75
Min, Max	0.3, 0.8	0.4, 0.8
Week 52		
n	13	14
Mean (SD)	0.63 (0.04)	0.62 (0.06)
Median	0.64	0.63
25th, 75th Percentile	0.61, 0.67	0.60, 0.67
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.001_mod_sub_sex_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	14
Mean (SD)	0.01 (0.12)	-0.04 (0.09)
Median	0.00	-0.02
25th, 75th Percentile	-0.03, 0.04	-0.05, 0.01
Min, Max	-0.2, 0.3	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.01 (-0.04, 0.02)	-0.03 (-0.06, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.07, 0.03)
P-value ^b		0.4300

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.001_mod_sub_sex_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.34 (-1.18, 0.50)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.001_mod_sub_sex_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Female		
Baseline		
n	18	15
Mean (SD)	0.67 (0.08)	0.68 (0.07)
Median	0.66	0.69
25th, 75th Percentile	0.60, 0.71	0.64, 0.74
Min, Max	0.6, 0.9	0.5, 0.8
Week 52		
n	18	15
Mean (SD)	0.63 (0.05)	0.68 (0.08)
Median	0.61	0.69
25th, 75th Percentile	0.60, 0.66	0.61, 0.72
Min, Max	0.5, 0.7	0.5, 0.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.001_mod_sub_sex_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	15
Mean (SD)	-0.04 (0.10)	-0.01 (0.10)
Median	-0.04	0.00
25th, 75th Percentile	-0.11, 0.04	-0.12, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.05 (-0.08, -0.01)	0.00 (-0.04, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		0.04 (-0.01, 0.09)
P-value ^b		0.0832

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.001_mod_sub_sex_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.70 (-0.09, 1.48)
P-value for interaction term, treatment * [Sex]		0.0495

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.001_mod_sub_sex_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	24	19
Mean (SD)	0.64 (0.10)	0.69 (0.08)
Median	0.64	0.68
25th, 75th Percentile	0.58, 0.70	0.64, 0.75
Min, Max	0.3, 0.9	0.5, 0.8
Week 52		
n	24	19
Mean (SD)	0.63 (0.05)	0.64 (0.06)
Median	0.62	0.65
25th, 75th Percentile	0.60, 0.65	0.60, 0.68
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.002_mod_sub_eth_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	24	19
Mean (SD)	-0.02 (0.12)	-0.05 (0.10)
Median	-0.01	-0.04
25th, 75th Percentile	-0.09, 0.04	-0.12, 0.02
Min, Max	-0.2, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.04 (-0.06, -0.01)	-0.02 (-0.05, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.02, 0.05)
P-value ^b		0.4853

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.002_mod_sub_eth_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.23 (-0.42, 0.89)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.002_mod_sub_eth_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Non-White		
Baseline		
n	7	10
Mean (SD)	0.66 (0.07)	0.65 (0.10)
Median	0.67	0.66
25th, 75th Percentile	0.60, 0.71	0.63, 0.73
Min, Max	0.6, 0.8	0.4, 0.8
Week 52		
n	7	10
Mean (SD)	0.63 (0.05)	0.67 (0.10)
Median	0.66	0.69
25th, 75th Percentile	0.61, 0.67	0.62, 0.72
Min, Max	0.5, 0.7	0.5, 0.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.002_mod_sub_eth_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	10
Mean (SD)	-0.03 (0.07)	0.02 (0.05)
Median	-0.03	0.02
25th, 75th Percentile	-0.09, 0.04	-0.01, 0.07
Min, Max	-0.1, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	-0.03 (-0.07, 0.01)	0.03 (0.00, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.06 (0.01, 0.11)
P-value ^b		0.0328

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.002_mod_sub_eth_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		1.47 (0.12, 2.77)
P-value for interaction term,treatment * [Ethnicity]		0.4083

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.002_mod_sub_eth_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	0.66 (0.09)	0.61 (0.10)
Median	0.67	0.64
25th, 75th Percentile	0.59, 0.72	0.55, 0.68
Min, Max	0.5, 0.7	0.4, 0.7
Week 52		
n	4	8
Mean (SD)	0.65 (0.05)	0.64 (0.08)
Median	0.65	0.66
25th, 75th Percentile	0.61, 0.69	0.60, 0.71
Min, Max	0.6, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.003_mod_sub_strat_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	-0.01 (0.04)	0.04 (0.06)
Median	-0.01	0.02
25th, 75th Percentile	-0.04, 0.02	0.00, 0.07
Min, Max	0.0, 0.0	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.02, 0.07)	0.02 (-0.01, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.05, 0.05)
P-value ^b		0.9171

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.003_mod_sub_strat_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.07 (-1.42, 1.28)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.003_mod_sub_strat_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	0.63 (0.07)	0.68 (0.05)
Median	0.63	0.67
25th, 75th Percentile	0.58, 0.67	0.64, 0.74
Min, Max	0.5, 0.8	0.6, 0.8
Week 52		
n	12	7
Mean (SD)	0.62 (0.06)	0.66 (0.06)
Median	0.62	0.67
25th, 75th Percentile	0.61, 0.66	0.62, 0.72
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.003_mod_sub_strat_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	-0.01 (0.09)	-0.02 (0.09)
Median	-0.01	-0.01
25th, 75th Percentile	-0.06, 0.04	-0.05, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.03 (-0.07, 0.01)	0.02 (-0.03, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (-0.01, 0.12)
P-value ^b		0.1161

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.003_mod_sub_strat_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.88 (-0.21, 1.94)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.4372

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.003_mod_sub_strat_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	6
Mean (SD)	0.64 (0.07)	0.64 (0.12)
Median	0.64	0.65
25th, 75th Percentile	0.59, 0.69	0.63, 0.74
Min, Max	0.5, 0.8	0.4, 0.8
Week 52		
n	12	6
Mean (SD)	0.64 (0.04)	0.63 (0.10)
Median	0.63	0.64
25th, 75th Percentile	0.61, 0.67	0.54, 0.72
Min, Max	0.6, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.004_mod_sub_agv_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	6
Mean (SD)	0.00 (0.08)	-0.01 (0.10)
Median	-0.01	0.01
25th, 75th Percentile	-0.03, 0.04	-0.01, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.05, 0.04)	-0.01 (-0.08, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.10, 0.07)
P-value ^b		0.7448

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.004_mod_sub_agv_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.19 (-1.29, 0.92)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.004_mod_sub_agv_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	19	23
Mean (SD)	0.65 (0.11)	0.68 (0.08)
Median	0.65	0.68
25th, 75th Percentile	0.59, 0.71	0.63, 0.75
Min, Max	0.3, 0.9	0.5, 0.8
Week 52		
n	19	23
Mean (SD)	0.62 (0.05)	0.66 (0.07)
Median	0.62	0.67
25th, 75th Percentile	0.59, 0.66	0.60, 0.69
Min, Max	0.5, 0.7	0.5, 0.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.004_mod_sub_agv_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	19	23
Mean (SD)	-0.03 (0.13)	-0.03 (0.09)
Median	-0.03	-0.01
25th, 75th Percentile	-0.10, 0.04	-0.10, 0.02
Min, Max	-0.2, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.04 (-0.07, -0.02)	-0.02 (-0.04, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.03 (-0.01, 0.07)
P-value ^b		0.1695

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.004_mod_sub_agv_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.4
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.47 (-0.20, 1.14)
P-value for interaction term,treatment * [Baseline AGV]		0.2436

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.004_mod_sub_agv_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	17	13
Mean (SD)	0.66 (0.08)	0.65 (0.10)
Median	0.64	0.64
25th, 75th Percentile	0.62, 0.70	0.63, 0.67
Min, Max	0.6, 0.9	0.4, 0.8
Week 52		
n	17	13
Mean (SD)	0.64 (0.04)	0.64 (0.08)
Median	0.64	0.65
25th, 75th Percentile	0.61, 0.66	0.60, 0.69
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.005_mod_sub_haz_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	17	13
Mean (SD)	-0.03 (0.09)	-0.01 (0.10)
Median	-0.02	0.02
25th, 75th Percentile	-0.09, 0.02	-0.01, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.03 (-0.06, 0.01)	-0.01 (-0.05, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.04, 0.07)
P-value ^b		0.5550

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.005_mod_sub_haz_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.5
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.24 (-0.56, 1.05)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.005_mod_sub_haz_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	16
Mean (SD)	0.63 (0.12)	0.69 (0.08)
Median	0.63	0.69
25th, 75th Percentile	0.57, 0.71	0.66, 0.76
Min, Max	0.3, 0.8	0.5, 0.8
Week 52		
n	14	16
Mean (SD)	0.62 (0.06)	0.66 (0.07)
Median	0.61	0.67
25th, 75th Percentile	0.58, 0.66	0.61, 0.70
Min, Max	0.5, 0.7	0.5, 0.8

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.005_mod_sub_haz_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	16
Mean (SD)	-0.01 (0.14)	-0.04 (0.09)
Median	-0.02	-0.04
25th, 75th Percentile	-0.07, 0.04	-0.11, 0.01
Min, Max	-0.2, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.03 (-0.07, 0.01)	-0.02 (-0.05, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.05, 0.07)
P-value ^b		0.6280

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.005_mod_sub_haz_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.101.5
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.22 (-0.67, 1.11)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.5178

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.101.005_mod_sub_haz_legrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	0.62 (0.06)	0.62 (0.11)
Median	0.63	0.64
25th, 75th Percentile	0.54, 0.67	0.57, 0.69
Min, Max	0.5, 0.7	0.4, 0.8
Week 52		
n	7	7
Mean (SD)	0.63 (0.05)	0.59 (0.06)
Median	0.64	0.62
25th, 75th Percentile	0.58, 0.67	0.54, 0.64
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.001_mod_sub_sex_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.1

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	0.01 (0.03)	-0.03 (0.09)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.04	-0.05, 0.02
Min, Max	0.0, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.06)	-0.03 (-0.08, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.11, 0.02)
P-value ^b		0.1531

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.001_mod_sub_sex_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.1

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.90 (-2.07, 0.32)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.001_mod_sub_sex_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	0.65 (0.08)	0.66 (0.06)
Median	0.64	0.66
25th, 75th Percentile	0.58, 0.71	0.63, 0.70
Min, Max	0.6, 0.8	0.5, 0.7
Week 52		
n	9	8
Mean (SD)	0.63 (0.06)	0.70 (0.02)
Median	0.61	0.71
25th, 75th Percentile	0.61, 0.66	0.68, 0.72
Min, Max	0.5, 0.7	0.7, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.001_mod_sub_sex_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.1

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	-0.02 (0.10)	0.05 (0.06)
Median	-0.03	0.04
25th, 75th Percentile	-0.10, 0.04	0.01, 0.07
Min, Max	-0.2, 0.2	0.0, 0.2
LS mean change from baseline (95% CI)	-0.02 (-0.05, 0.01)	0.05 (0.02, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.07 (0.02, 0.12)
P-value ^b		0.0065

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.001_mod_sub_sex_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.1

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		1.83 (0.49, 3.11)
P-value for interaction term,treatment * [Sex]		0.0024

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.001_mod_sub_sex_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	0.63 (0.08)	0.66 (0.07)
Median	0.63	0.66
25th, 75th Percentile	0.57, 0.66	0.63, 0.70
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	13	8
Mean (SD)	0.63 (0.05)	0.66 (0.06)
Median	0.62	0.67
25th, 75th Percentile	0.61, 0.66	0.64, 0.71
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.002_mod_sub_eth_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	0.00 (0.08)	0.01 (0.11)
Median	0.00	0.02
25th, 75th Percentile	-0.03, 0.04	-0.03, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.01 (-0.04, 0.02)	0.03 (-0.01, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.03 (-0.01, 0.08)
P-value ^b		0.1519

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.002_mod_sub_eth_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.70 (-0.25, 1.64)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.002_mod_sub_eth_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	0.66 (0.04)	0.62 (0.11)
Median	0.67	0.64
25th, 75th Percentile	0.62, 0.70	0.57, 0.69
Min, Max	0.6, 0.7	0.4, 0.7
Week 52		
n	3	7
Mean (SD)	0.62 (0.08)	0.64 (0.09)
Median	0.66	0.64
25th, 75th Percentile	0.52, 0.67	0.59, 0.72
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.002_mod_sub_eth_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	-0.05 (0.05)	0.02 (0.04)
Median	-0.03	0.01
25th, 75th Percentile	-0.10, -0.01	-0.01, 0.07
Min, Max	-0.1, 0.0	-0.1, 0.1
LS mean change from baseline (95% CI)	-0.17 (-0.32, -0.02)	0.07 (0.00, 0.13)
Difference in LS mean change from baseline (95% CI) ^a		0.24 (0.03, 0.44)
P-value ^b		0.0345

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.002_mod_sub_eth_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		10.33 (0.61, 19.72)
P-value for interaction term,treatment * [Ethnicity]		0.7559

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.002_mod_sub_eth_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.3

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	0.66 (0.09)	0.61 (0.10)
Median	0.67	0.64
25th, 75th Percentile	0.59, 0.72	0.55, 0.68
Min, Max	0.5, 0.7	0.4, 0.7
Week 52		
n	4	8
Mean (SD)	0.65 (0.05)	0.64 (0.08)
Median	0.65	0.66
25th, 75th Percentile	0.61, 0.69	0.60, 0.71
Min, Max	0.6, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.003_mod_sub_strat_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.3

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	-0.01 (0.04)	0.04 (0.06)
Median	-0.01	0.02
25th, 75th Percentile	-0.04, 0.02	0.00, 0.07
Min, Max	0.0, 0.0	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.02, 0.07)	0.02 (-0.01, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.05, 0.05)
P-value ^b		0.9171

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.003_mod_sub_strat_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.3

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.07 (-1.42, 1.28)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.003_mod_sub_strat_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.3

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	0.63 (0.07)	0.68 (0.05)
Median	0.63	0.67
25th, 75th Percentile	0.58, 0.67	0.64, 0.74
Min, Max	0.5, 0.8	0.6, 0.8
Week 52		
n	12	7
Mean (SD)	0.62 (0.06)	0.66 (0.06)
Median	0.62	0.67
25th, 75th Percentile	0.61, 0.66	0.62, 0.72
Min, Max	0.5, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.003_mod_sub_strat_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.3

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	-0.01 (0.09)	-0.02 (0.09)
Median	-0.01	-0.01
25th, 75th Percentile	-0.06, 0.04	-0.05, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.03 (-0.07, 0.01)	0.02 (-0.03, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (-0.01, 0.12)
P-value ^b		0.1161

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.003_mod_sub_strat_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.3

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.88 (-0.21, 1.94)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.4372

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.003_mod_sub_strat_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
Baseline		
n	11	6
Mean (SD)	0.65 (0.08)	0.64 (0.12)
Median	0.64	0.65
25th, 75th Percentile	0.57, 0.71	0.63, 0.74
Min, Max	0.5, 0.8	0.4, 0.8
Week 52		
n	11	6
Mean (SD)	0.64 (0.05)	0.63 (0.10)
Median	0.64	0.64
25th, 75th Percentile	0.61, 0.67	0.54, 0.72
Min, Max	0.6, 0.7	0.5, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.004_mod_sub_agv_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	0.00 (0.09)	-0.01 (0.10)
Median	-0.01	0.01
25th, 75th Percentile	-0.04, 0.04	-0.01, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.05, 0.05)	-0.01 (-0.08, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.10, 0.07)
P-value ^b		0.7448

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.004_mod_sub_agv_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.19 (-1.29, 0.92)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.004_mod_sub_agv_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	0.62 (0.06)	0.64 (0.06)
Median	0.62	0.67
25th, 75th Percentile	0.58, 0.64	0.61, 0.69
Min, Max	0.5, 0.7	0.5, 0.7
Week 52		
n	5	9
Mean (SD)	0.60 (0.06)	0.67 (0.05)
Median	0.62	0.68
25th, 75th Percentile	0.54, 0.64	0.64, 0.69
Min, Max	0.5, 0.7	0.6, 0.7

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.004_mod_sub_agv_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.4
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	-0.02 (0.05)	0.03 (0.07)
Median	0.00	0.02
25th, 75th Percentile	-0.03, 0.00	-0.01, 0.06
Min, Max	-0.1, 0.0	-0.1, 0.2
LS mean change from baseline (95% CI)	-0.02 (-0.06, 0.02)	0.03 (0.00, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (-0.01, 0.11)
P-value ^b		0.1013

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.004_mod_sub_agv_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.4.102.4
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for
 BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		1.33 (-0.25, 2.83)
P-value for interaction term,treatment * [Baseline AGV]		0.2399

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to knee to heel length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.004.102.004_mod_sub_agv_legrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	14
Mean (SD)	1.05 (0.21)	1.08 (0.19)
Median	1.05	1.09
25th, 75th Percentile	0.99, 1.10	0.95, 1.23
Min, Max	0.6, 1.4	0.7, 1.4
Week 52		
n	13	14
Mean (SD)	1.05 (0.13)	1.00 (0.09)
Median	1.05	1.00
25th, 75th Percentile	1.00, 1.08	0.97, 1.06
Min, Max	0.8, 1.4	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.001_mod_sub_sex_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	14
Mean (SD)	0.00 (0.24)	-0.08 (0.16)
Median	0.02	-0.07
25th, 75th Percentile	-0.01, 0.07	-0.19, 0.00
Min, Max	-0.6, 0.5	-0.4, 0.2
LS mean change from baseline (95% CI)	-0.01 (-0.07, 0.06)	-0.07 (-0.13, -0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.06 (-0.16, 0.03)
P-value ^b		0.1699

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.001_mod_sub_sex_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.60 (-1.43, 0.25)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.001_mod_sub_sex_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Female		
Baseline		
n	18	15
Mean (SD)	1.06 (0.17)	1.06 (0.14)
Median	1.01	1.04
25th, 75th Percentile	0.96, 1.13	0.99, 1.17
Min, Max	0.9, 1.5	0.8, 1.3
Week 52		
n	18	15
Mean (SD)	1.00 (0.09)	1.09 (0.12)
Median	1.00	1.10
25th, 75th Percentile	0.93, 1.02	1.01, 1.14
Min, Max	0.9, 1.3	0.9, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.001_mod_sub_sex_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	15
Mean (SD)	-0.07 (0.18)	0.03 (0.19)
Median	-0.06	0.02
25th, 75th Percentile	-0.13, 0.04	-0.13, 0.21
Min, Max	-0.5, 0.2	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.06 (-0.11, 0.00)	0.02 (-0.04, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.08 (0.00, 0.16)
P-value ^b		0.0601

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.001_mod_sub_sex_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.75 (-0.03, 1.53)
P-value for interaction term, treatment * [Sex]		0.0074

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.001_mod_sub_sex_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	24	19
Mean (SD)	1.06 (0.21)	1.07 (0.14)
Median	1.02	1.06
25th, 75th Percentile	0.96, 1.18	0.99, 1.20
Min, Max	0.6, 1.5	0.8, 1.3
Week 52		
n	24	19
Mean (SD)	1.02 (0.13)	1.03 (0.09)
Median	1.01	1.02
25th, 75th Percentile	0.93, 1.05	0.99, 1.10
Min, Max	0.8, 1.4	0.9, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.002_mod_sub_eth_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	24	19
Mean (SD)	-0.04 (0.23)	-0.04 (0.18)
Median	-0.02	-0.08
25th, 75th Percentile	-0.11, 0.06	-0.19, 0.05
Min, Max	-0.6, 0.5	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.05 (-0.10, -0.01)	-0.03 (-0.08, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.05, 0.09)
P-value ^b		0.5421

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.002_mod_sub_eth_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.2
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.19 (-0.43, 0.81)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.002_mod_sub_eth_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Non-White		
Baseline		
n	7	10
Mean (SD)	1.03 (0.09)	1.06 (0.21)
Median	1.06	1.07
25th, 75th Percentile	0.97, 1.10	0.95, 1.17
Min, Max	0.9, 1.2	0.7, 1.4
Week 52		
n	7	10
Mean (SD)	1.02 (0.06)	1.07 (0.15)
Median	1.00	1.08
25th, 75th Percentile	1.00, 1.08	0.98, 1.16
Min, Max	0.9, 1.1	0.8, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.002_mod_sub_eth_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	10
Mean (SD)	-0.01 (0.08)	0.01 (0.18)
Median	0.00	0.00
25th, 75th Percentile	-0.11, 0.02	-0.06, 0.12
Min, Max	-0.1, 0.1	-0.4, 0.3
LS mean change from baseline (95% CI)	-0.04 (-0.11, 0.04)	0.02 (-0.04, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.06 (-0.04, 0.16)
P-value ^b		0.2071

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.002_mod_sub_eth_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.80 (-0.43, 1.99)
P-value for interaction term,treatment * [Ethnicity]		0.6993

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.002_mod_sub_eth_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	1.01 (0.12)	0.96 (0.22)
Median	1.05	0.94
25th, 75th Percentile	0.93, 1.09	0.83, 0.99
Min, Max	0.8, 1.1	0.7, 1.4
Week 52		
n	4	8
Mean (SD)	1.03 (0.08)	1.04 (0.14)
Median	1.05	1.07
25th, 75th Percentile	0.98, 1.08	0.94, 1.14
Min, Max	0.9, 1.1	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.003_mod_sub_strat_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	0.01 (0.05)	0.08 (0.24)
Median	0.01	0.09
25th, 75th Percentile	-0.02, 0.05	-0.03, 0.28
Min, Max	0.0, 0.1	-0.4, 0.3
LS mean change from baseline (95% CI)	0.07 (-0.04, 0.17)	0.06 (-0.02, 0.13)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.14, 0.12)
P-value ^b		0.8636

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.003_mod_sub_strat_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.11 (-1.36, 1.14)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.003_mod_sub_strat_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	1.06 (0.14)	1.09 (0.09)
Median	1.03	1.06
25th, 75th Percentile	0.98, 1.14	1.01, 1.17
Min, Max	0.9, 1.3	1.0, 1.2
Week 52		
n	12	7
Mean (SD)	1.05 (0.15)	1.05 (0.07)
Median	1.02	1.01
25th, 75th Percentile	0.93, 1.12	1.00, 1.11
Min, Max	0.9, 1.4	1.0, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.003_mod_sub_strat_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	-0.01 (0.13)	-0.04 (0.11)
Median	0.01	0.00
25th, 75th Percentile	-0.07, 0.05	-0.12, 0.04
Min, Max	-0.3, 0.2	-0.2, 0.0
LS mean change from baseline (95% CI)	-0.01 (-0.09, 0.06)	-0.03 (-0.13, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.14, 0.11)
P-value ^b		0.7710

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.003_mod_sub_strat_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.14 (-1.08, 0.80)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.7515

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.003_mod_sub_strat_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	6
Mean (SD)	1.06 (0.14)	1.03 (0.19)
Median	1.04	1.04
25th, 75th Percentile	0.98, 1.15	0.98, 1.17
Min, Max	0.8, 1.3	0.7, 1.2
Week 52		
n	12	6
Mean (SD)	1.07 (0.15)	1.01 (0.12)
Median	1.04	1.00
25th, 75th Percentile	0.93, 1.15	0.98, 1.11
Min, Max	0.9, 1.4	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.004_mod_sub_agv_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	6
Mean (SD)	0.01 (0.13)	-0.02 (0.12)
Median	0.01	0.00
25th, 75th Percentile	-0.06, 0.08	-0.01, 0.05
Min, Max	-0.3, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.07, 0.10)	-0.03 (-0.15, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.20, 0.11)
P-value ^b		0.5206

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.004_mod_sub_agv_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.37 (-1.46, 0.74)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.004_mod_sub_agv_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	19	23
Mean (SD)	1.05 (0.21)	1.08 (0.16)
Median	1.03	1.08
25th, 75th Percentile	0.97, 1.13	0.95, 1.20
Min, Max	0.6, 1.5	0.8, 1.4
Week 52		
n	19	23
Mean (SD)	0.99 (0.07)	1.05 (0.11)
Median	1.00	1.06
25th, 75th Percentile	0.94, 1.05	0.99, 1.12
Min, Max	0.8, 1.1	0.9, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.004_mod_sub_agv_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	19	23
Mean (SD)	-0.06 (0.24)	-0.02 (0.20)
Median	-0.02	-0.06
25th, 75th Percentile	-0.13, 0.04	-0.13, 0.13
Min, Max	-0.6, 0.5	-0.4, 0.3
LS mean change from baseline (95% CI)	-0.07 (-0.11, -0.03)	-0.02 (-0.06, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (0.00, 0.11)
P-value ^b		0.0710

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.004_mod_sub_agv_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.4
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.61 (-0.05, 1.26)
P-value for interaction term,treatment * [Baseline AGV]		0.1345

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.004_mod_sub_agv_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	17	13
Mean (SD)	1.08 (0.13)	1.03 (0.14)
Median	1.06	1.01
25th, 75th Percentile	1.01, 1.16	0.98, 1.13
Min, Max	0.9, 1.3	0.7, 1.2
Week 52		
n	17	13
Mean (SD)	1.06 (0.13)	1.01 (0.11)
Median	1.04	1.00
25th, 75th Percentile	0.94, 1.10	0.97, 1.08
Min, Max	0.9, 1.4	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.005_mod_sub_haz_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	17	13
Mean (SD)	-0.03 (0.13)	-0.02 (0.15)
Median	-0.01	0.00
25th, 75th Percentile	-0.10, 0.04	-0.06, 0.05
Min, Max	-0.3, 0.2	-0.3, 0.3
LS mean change from baseline (95% CI)	-0.02 (-0.08, 0.04)	-0.04 (-0.11, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.11, 0.08)
P-value ^b		0.6861

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.005_mod_sub_haz_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.17 (-0.97, 0.64)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.005_mod_sub_haz_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	16
Mean (SD)	1.02 (0.24)	1.10 (0.17)
Median	1.00	1.09
25th, 75th Percentile	0.89, 1.08	0.97, 1.24
Min, Max	0.6, 1.5	0.8, 1.4
Week 52		
n	14	16
Mean (SD)	0.98 (0.07)	1.07 (0.12)
Median	1.00	1.07
25th, 75th Percentile	0.91, 1.03	1.01, 1.13
Min, Max	0.8, 1.1	0.9, 1.3

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.005_mod_sub_haz_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	16
Mean (SD)	-0.05 (0.28)	-0.02 (0.21)
Median	-0.01	-0.08
25th, 75th Percentile	-0.12, 0.07	-0.16, 0.15
Min, Max	-0.6, 0.5	-0.4, 0.3
LS mean change from baseline (95% CI)	-0.09 (-0.14, -0.03)	0.01 (-0.04, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.10 (0.02, 0.17)
P-value ^b		0.0155

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.005_mod_sub_haz_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.101.5
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		1.07 (0.20, 1.92)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.0557

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.101.005_mod_sub_haz_legtrt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	1.05 (0.14)	1.06 (0.24)
Median	1.05	1.01
25th, 75th Percentile	0.99, 1.10	0.95, 1.25
Min, Max	0.8, 1.3	0.7, 1.4
Week 52		
n	7	7
Mean (SD)	1.09 (0.15)	0.96 (0.08)
Median	1.06	0.98
25th, 75th Percentile	1.04, 1.10	0.89, 1.00
Min, Max	0.9, 1.4	0.8, 1.1

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.001_mod_sub_sex_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	0.04 (0.04)	-0.10 (0.17)
Median	0.03	-0.05
25th, 75th Percentile	0.00, 0.07	-0.25, 0.00
Min, Max	0.0, 0.1	-0.4, 0.1
LS mean change from baseline (95% CI)	0.03 (-0.04, 0.11)	-0.09 (-0.17, -0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.12 (-0.23, -0.01)
P-value ^b		0.0314

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.001_mod_sub_sex_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.1

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-1.49 (-2.79, -0.13)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.001_mod_sub_sex_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	1.05 (0.13)	0.98 (0.12)
Median	1.01	1.00
25th, 75th Percentile	0.96, 1.08	0.90, 1.05
Min, Max	0.9, 1.3	0.8, 1.2
Week 52		
n	9	8
Mean (SD)	1.01 (0.12)	1.12 (0.07)
Median	0.94	1.12
25th, 75th Percentile	0.93, 1.04	1.08, 1.16
Min, Max	0.9, 1.3	1.0, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.001_mod_sub_sex_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	-0.04 (0.15)	0.14 (0.14)
Median	-0.04	0.06
25th, 75th Percentile	-0.07, 0.04	0.03, 0.28
Min, Max	-0.3, 0.2	0.0, 0.3
LS mean change from baseline (95% CI)	0.00 (-0.08, 0.07)	0.10 (0.02, 0.18)
Difference in LS mean change from baseline (95% CI) ^a		0.11 (-0.01, 0.22)
P-value ^b		0.0712

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.001_mod_sub_sex_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.1

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		1.10 (-0.09, 2.25)
P-value for interaction term,treatment * [Sex]		0.0008

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.001_mod_sub_sex_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	1.05 (0.14)	1.02 (0.13)
Median	1.01	1.02
25th, 75th Percentile	0.96, 1.08	0.96, 1.07
Min, Max	0.8, 1.3	0.8, 1.2
Week 52		
n	13	8
Mean (SD)	1.05 (0.15)	1.07 (0.09)
Median	1.04	1.04
25th, 75th Percentile	0.94, 1.06	1.00, 1.13
Min, Max	0.9, 1.4	1.0, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.002_mod_sub_eth_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	0.00 (0.12)	0.05 (0.20)
Median	0.03	0.03
25th, 75th Percentile	-0.04, 0.06	-0.07, 0.17
Min, Max	-0.3, 0.2	-0.2, 0.3
LS mean change from baseline (95% CI)	0.01 (-0.07, 0.09)	0.04 (-0.06, 0.14)
Difference in LS mean change from baseline (95% CI) ^a		0.03 (-0.10, 0.16)
P-value ^b		0.6349

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.002_mod_sub_eth_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.2

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.22 (-0.68, 1.11)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.002_mod_sub_eth_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	1.07 (0.02)	1.02 (0.24)
Median	1.06	0.98
25th, 75th Percentile	1.06, 1.10	0.87, 1.17
Min, Max	1.1, 1.1	0.7, 1.4
Week 52		
n	3	7
Mean (SD)	1.03 (0.09)	1.02 (0.13)
Median	1.08	1.06
25th, 75th Percentile	0.92, 1.10	0.89, 1.13
Min, Max	0.9, 1.1	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.002_mod_sub_eth_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.2
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	-0.04 (0.08)	0.00 (0.20)
Median	0.00	0.00
25th, 75th Percentile	-0.13, 0.02	-0.05, 0.12
Min, Max	-0.1, 0.0	-0.4, 0.3
LS mean change from baseline (95% CI)	-0.24 (-0.66, 0.18)	0.09 (-0.10, 0.28)
Difference in LS mean change from baseline (95% CI) ^a		0.33 (-0.26, 0.92)
P-value ^b		0.1762

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.002_mod_sub_eth_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.2
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Ethnicity for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		4.69 (-1.87, 10.78)
P-value for interaction term,treatment * [Ethnicity]		0.5923

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.002_mod_sub_eth_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	1.01 (0.12)	0.96 (0.22)
Median	1.05	0.94
25th, 75th Percentile	0.93, 1.09	0.83, 0.99
Min, Max	0.8, 1.1	0.7, 1.4
Week 52		
n	4	8
Mean (SD)	1.03 (0.08)	1.04 (0.14)
Median	1.05	1.07
25th, 75th Percentile	0.98, 1.08	0.94, 1.14
Min, Max	0.9, 1.1	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.5.102.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	0.01 (0.05)	0.08 (0.24)
Median	0.01	0.09
25th, 75th Percentile	-0.02, 0.05	-0.03, 0.28
Min, Max	0.0, 0.1	-0.4, 0.3
LS mean change from baseline (95% CI)	0.07 (-0.04, 0.17)	0.06 (-0.02, 0.13)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.14, 0.12)
P-value ^b		0.8636

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.003_mod_sub_strat_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.11 (-1.36, 1.14)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.003_mod_sub_strat_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	1.06 (0.14)	1.09 (0.09)
Median	1.03	1.06
25th, 75th Percentile	0.98, 1.14	1.01, 1.17
Min, Max	0.9, 1.3	1.0, 1.2
Week 52		
n	12	7
Mean (SD)	1.05 (0.15)	1.05 (0.07)
Median	1.02	1.01
25th, 75th Percentile	0.93, 1.12	1.00, 1.11
Min, Max	0.9, 1.4	1.0, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.003_mod_sub_strat_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by [Cohort 1]Age stratum for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	-0.01 (0.13)	-0.04 (0.11)
Median	0.01	0.00
25th, 75th Percentile	-0.07, 0.05	-0.12, 0.04
Min, Max	-0.3, 0.2	-0.2, 0.0
LS mean change from baseline (95% CI)	-0.01 (-0.09, 0.06)	-0.03 (-0.13, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.14, 0.11)
P-value ^b		0.7710

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.003_mod_sub_strat_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.3
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.14 (-1.08, 0.80)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.7515

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.5.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
Baseline		
n	11	6
Mean (SD)	1.07 (0.15)	1.03 (0.19)
Median	1.05	1.04
25th, 75th Percentile	0.96, 1.22	0.98, 1.17
Min, Max	0.8, 1.3	0.7, 1.2
Week 52		
n	11	6
Mean (SD)	1.06 (0.16)	1.01 (0.12)
Median	1.04	1.00
25th, 75th Percentile	0.93, 1.17	0.98, 1.11
Min, Max	0.9, 1.4	0.8, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.004_mod_sub_agv_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	0.00 (0.13)	-0.02 (0.12)
Median	-0.01	0.00
25th, 75th Percentile	-0.07, 0.07	-0.01, 0.05
Min, Max	-0.3, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.08, 0.09)	-0.04 (-0.16, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.20, 0.11)
P-value ^b		0.5206

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.004_mod_sub_agv_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.37 (-1.46, 0.74)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.004_mod_sub_agv_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	1.02 (0.08)	1.01 (0.18)
Median	1.03	0.99
25th, 75th Percentile	0.99, 1.06	0.93, 1.04
Min, Max	0.9, 1.1	0.8, 1.4
Week 52		
n	5	9
Mean (SD)	1.01 (0.08)	1.07 (0.10)
Median	1.05	1.08
25th, 75th Percentile	0.94, 1.06	1.01, 1.13
Min, Max	0.9, 1.1	0.9, 1.2

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.004_mod_sub_agv_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	0.00 (0.08)	0.05 (0.23)
Median	0.03	0.04
25th, 75th Percentile	0.00, 0.04	-0.05, 0.26
Min, Max	-0.1, 0.1	-0.4, 0.3
LS mean change from baseline (95% CI)	0.04 (-0.06, 0.14)	0.03 (-0.04, 0.10)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.14, 0.12)
P-value ^b		0.8412

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.005.102.004_mod_sub_agv_legtrt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.5.102.4

Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.14 (-1.47, 1.20)
P-value for interaction term,treatment * [Baseline AGV]		0.5797

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline upper leg length to tibial leg length ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Male		
Baseline		
n	13	13
Mean (SD)	0.88 (0.04)	0.89 (0.03)
Median	0.89	0.88
25th, 75th Percentile	0.84, 0.90	0.87, 0.93
Min, Max	0.8, 0.9	0.9, 0.9
Week 52		
n	13	13
Mean (SD)	0.88 (0.03)	0.88 (0.03)
Median	0.88	0.88
25th, 75th Percentile	0.87, 0.90	0.86, 0.90
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	13	13
Mean (SD)	0.01 (0.02)	-0.01 (0.02)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.02	-0.02, -0.01
Min, Max	0.0, 0.1	0.0, 0.0
LS mean change from baseline (95% CI)	0.01 (0.00, 0.02)	-0.01 (-0.02, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.03, 0.00)
P-value ^b		0.0113

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-1.21 (-2.12, -0.27)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Female		
Baseline		
n	18	15
Mean (SD)	0.88 (0.03)	0.86 (0.05)
Median	0.89	0.87
25th, 75th Percentile	0.86, 0.91	0.84, 0.89
Min, Max	0.8, 0.9	0.7, 0.9
Week 52		
n	18	15
Mean (SD)	0.87 (0.03)	0.86 (0.06)
Median	0.87	0.87
25th, 75th Percentile	0.86, 0.90	0.85, 0.89
Min, Max	0.8, 0.9	0.7, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	18	15
Mean (SD)	-0.01 (0.02)	0.00 (0.09)
Median	-0.01	-0.01
25th, 75th Percentile	-0.03, 0.02	-0.03, 0.02
Min, Max	-0.1, 0.0	-0.2, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.01, 0.03)	-0.03 (-0.05, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.04 (-0.07, 0.00)
P-value ^b		0.0593

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.82 (-1.65, 0.03)
P-value for interaction term,treatment * [Sex]		0.5379

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
White		
Baseline		
n	24	18
Mean (SD)	0.88 (0.03)	0.87 (0.03)
Median	0.88	0.87
25th, 75th Percentile	0.85, 0.91	0.85, 0.89
Min, Max	0.8, 0.9	0.8, 0.9
Week 52		
n	24	18
Mean (SD)	0.88 (0.03)	0.86 (0.06)
Median	0.87	0.86
25th, 75th Percentile	0.86, 0.90	0.85, 0.89
Min, Max	0.8, 0.9	0.7, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	24	18
Mean (SD)	0.00 (0.03)	-0.01 (0.06)
Median	0.00	-0.01
25th, 75th Percentile	-0.02, 0.02	-0.02, 0.01
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.02)	-0.02 (-0.04, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.04, 0.00)
P-value ^b		0.1169

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.52 (-1.15, 0.13)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Non-White		
Baseline		
n	7	10
Mean (SD)	0.89 (0.02)	0.88 (0.06)
Median	0.90	0.90
25th, 75th Percentile	0.87, 0.90	0.88, 0.91
Min, Max	0.9, 0.9	0.7, 0.9
Week 52		
n	7	10
Mean (SD)	0.88 (0.02)	0.89 (0.03)
Median	0.88	0.88
25th, 75th Percentile	0.88, 0.89	0.87, 0.92
Min, Max	0.9, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.006.101.002_mod_sub_eth_armsphgt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.6.101.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	7	10
Mean (SD)	-0.01 (0.02)	0.00 (0.07)
Median	-0.01	-0.02
25th, 75th Percentile	-0.02, 0.02	-0.03, -0.01
Min, Max	0.0, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.04, 0.03)	0.00 (-0.03, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.05, 0.05)
P-value ^b		0.9630

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.03 (-1.24, 1.30)
P-value for interaction term,treatment * [Ethnicity]		0.6712

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	0.90 (0.00)	0.89 (0.03)
Median	0.90	0.89
25th, 75th Percentile	0.90, 0.91	0.87, 0.92
Min, Max	0.9, 0.9	0.8, 0.9
Week 52		
n	4	8
Mean (SD)	0.91 (0.02)	0.90 (0.03)
Median	0.91	0.89
25th, 75th Percentile	0.89, 0.93	0.87, 0.92
Min, Max	0.9, 0.9	0.9, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	4	8
Mean (SD)	0.00 (0.02)	0.00 (0.04)
Median	0.01	-0.01
25th, 75th Percentile	-0.02, 0.02	-0.03, 0.01
Min, Max	0.0, 0.0	0.0, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.02, 0.05)	0.00 (-0.03, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.2910

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.75 (-2.05, 0.61)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	0.88 (0.03)	0.85 (0.07)
Median	0.87	0.86
25th, 75th Percentile	0.86, 0.90	0.81, 0.93
Min, Max	0.8, 0.9	0.7, 0.9
Week 52		
n	12	7
Mean (SD)	0.88 (0.03)	0.87 (0.05)
Median	0.87	0.86
25th, 75th Percentile	0.85, 0.90	0.83, 0.91
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	7
Mean (SD)	0.00 (0.02)	0.02 (0.08)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.01	-0.02, 0.00
Min, Max	-0.1, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.01, 0.03)	0.00 (-0.03, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.04, 0.03)
P-value ^b		0.7000

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.20 (-1.19, 0.80)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.8197

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
Baseline		
n	12	6
Mean (SD)	0.87 (0.03)	0.87 (0.09)
Median	0.87	0.90
25th, 75th Percentile	0.85, 0.90	0.81, 0.93
Min, Max	0.8, 0.9	0.7, 0.9
Week 52		
n	12	6
Mean (SD)	0.88 (0.03)	0.89 (0.05)
Median	0.86	0.91
25th, 75th Percentile	0.85, 0.91	0.85, 0.92
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.006.101.004_mod_sub_agv_armsphgt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.6.101.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	12	6
Mean (SD)	0.00 (0.02)	0.02 (0.09)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.02	-0.02, 0.00
Min, Max	-0.1, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.02)	0.02 (0.00, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.01, 0.05)
P-value ^b		0.2114

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.77 (-0.42, 1.92)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
Baseline		
n	19	22
Mean (SD)	0.89 (0.03)	0.88 (0.03)
Median	0.90	0.88
25th, 75th Percentile	0.86, 0.91	0.86, 0.90
Min, Max	0.8, 0.9	0.8, 0.9
Week 52		
n	19	22
Mean (SD)	0.88 (0.02)	0.86 (0.05)
Median	0.88	0.87
25th, 75th Percentile	0.87, 0.90	0.86, 0.88
Min, Max	0.8, 0.9	0.7, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	19	22
Mean (SD)	-0.01 (0.03)	-0.02 (0.05)
Median	-0.01	-0.01
25th, 75th Percentile	-0.02, 0.02	-0.03, 0.00
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.02)	-0.02 (-0.04, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.05, 0.01)
P-value ^b		0.1364

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.50 (-1.15, 0.16)
P-value for interaction term, treatment * [Baseline AGV]		0.1979

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.5
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
<= -4		
Baseline		
n	17	13
Mean (SD)	0.87 (0.03)	0.88 (0.06)
Median	0.88	0.88
25th, 75th Percentile	0.85, 0.90	0.86, 0.93
Min, Max	0.8, 0.9	0.7, 0.9
Week 52		
n	17	13
Mean (SD)	0.87 (0.02)	0.88 (0.04)
Median	0.88	0.87
25th, 75th Percentile	0.86, 0.89	0.86, 0.91
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.006.101.005_mod_sub_haz_armsphgt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.6.101.5
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	17	13
Mean (SD)	0.00 (0.02)	0.00 (0.06)
Median	0.00	-0.02
25th, 75th Percentile	-0.01, 0.02	-0.02, -0.01
Min, Max	-0.1, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.02)	0.00 (-0.02, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.03, 0.03)
P-value ^b		0.9430

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.5
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		0.03 (-0.79, 0.85)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.5
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
> -4		
Baseline		
n	14	15
Mean (SD)	0.89 (0.03)	0.88 (0.02)
Median	0.90	0.87
25th, 75th Percentile	0.87, 0.91	0.85, 0.90
Min, Max	0.8, 0.9	0.8, 0.9
Week 52		
n	14	15
Mean (SD)	0.88 (0.03)	0.86 (0.06)
Median	0.88	0.87
25th, 75th Percentile	0.87, 0.91	0.85, 0.89
Min, Max	0.8, 0.9	0.7, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.006.101.005_mod_sub_haz_armsphgt_ov_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.6.101.5
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
Change from baseline		
n	14	15
Mean (SD)	-0.01 (0.03)	-0.02 (0.07)
Median	-0.01	-0.01
25th, 75th Percentile	-0.03, 0.02	-0.03, 0.01
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.03)	-0.02 (-0.05, 0.00)
Difference in LS mean change from baseline (95% CI) ^c		-0.03 (-0.06, 0.01)
P-value ^b		0.1983

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.101.5
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall), by Baseline Height Z-Score for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline Height Z-Score Arm Span to Standing Height Ratio	Placebo (N=32)	Vosoritide (N=32)
SMD (95% CI) ^c		-0.54 (-1.35, 0.28)
P-value for interaction term, treatment * [Baseline Height Z-Score]		0.1850

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Male		
Baseline		
n	7	7
Mean (SD)	0.88 (0.04)	0.91 (0.03)
Median	0.90	0.93
25th, 75th Percentile	0.84, 0.91	0.88, 0.94
Min, Max	0.8, 0.9	0.9, 0.9
Week 52		
n	7	7
Mean (SD)	0.89 (0.04)	0.90 (0.03)
Median	0.88	0.90
25th, 75th Percentile	0.85, 0.93	0.87, 0.92
Min, Max	0.8, 0.9	0.9, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	7	7
Mean (SD)	0.00 (0.02)	-0.02 (0.01)
Median	0.00	-0.02
25th, 75th Percentile	-0.01, 0.03	-0.02, -0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.02)	-0.02 (-0.03, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.05, 0.00)
P-value ^b		0.0775

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-1.32 (-2.72, 0.14)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Female		
Baseline		
n	9	8
Mean (SD)	0.88 (0.02)	0.84 (0.05)
Median	0.88	0.85
25th, 75th Percentile	0.86, 0.90	0.82, 0.88
Min, Max	0.9, 0.9	0.7, 0.9
Week 52		
n	9	8
Mean (SD)	0.88 (0.03)	0.87 (0.05)
Median	0.88	0.87
25th, 75th Percentile	0.86, 0.90	0.84, 0.91
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.006.102.001_mod_sub_sex_armsphgt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.6.102.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	9	8
Mean (SD)	0.00 (0.02)	0.03 (0.08)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.02	-0.02, 0.06
Min, Max	-0.1, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.04 (0.02, 0.07)	-0.02 (-0.05, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.11, -0.02)
P-value ^b		0.0105

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Sex for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Sex Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-2.33 (-4.06, -0.53)
P-value for interaction term, treatment * [Sex]		0.4371

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
White		
Baseline		
n	13	8
Mean (SD)	0.89 (0.03)	0.87 (0.04)
Median	0.90	0.86
25th, 75th Percentile	0.86, 0.90	0.84, 0.90
Min, Max	0.8, 0.9	0.8, 0.9
Week 52		
n	13	8
Mean (SD)	0.89 (0.03)	0.87 (0.05)
Median	0.90	0.87
25th, 75th Percentile	0.86, 0.91	0.84, 0.91
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	13	8
Mean (SD)	0.00 (0.02)	0.00 (0.04)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.02	-0.02, 0.01
Min, Max	-0.1, 0.0	0.0, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.01, 0.02)	0.00 (-0.02, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.03, 0.01)
P-value ^b		0.4732

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.35 (-1.29, 0.60)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Non-White		
Baseline		
n	3	7
Mean (SD)	0.88 (0.02)	0.88 (0.07)
Median	0.87	0.90
25th, 75th Percentile	0.86, 0.90	0.88, 0.93
Min, Max	0.9, 0.9	0.7, 0.9
Week 52		
n	3	7
Mean (SD)	0.87 (0.02)	0.89 (0.03)
Median	0.88	0.88
25th, 75th Percentile	0.85, 0.88	0.87, 0.92
Min, Max	0.9, 0.9	0.9, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	3	7
Mean (SD)	0.00 (0.02)	0.01 (0.08)
Median	-0.01	-0.02
25th, 75th Percentile	-0.02, 0.02	-0.03, 0.00
Min, Max	0.0, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.13 (-0.13, 0.38)	-0.04 (-0.15, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.17 (-0.52, 0.19)
P-value ^b		0.2294

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.2
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Ethnicity Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-6.11 (-15.00, 3.45)
P-value for interaction term,treatment * [Ethnicity]		0.3448

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
Baseline		
n	4	8
Mean (SD)	0.90 (0.00)	0.89 (0.03)
Median	0.90	0.89
25th, 75th Percentile	0.90, 0.91	0.87, 0.92
Min, Max	0.9, 0.9	0.8, 0.9
Week 52		
n	4	8
Mean (SD)	0.91 (0.02)	0.90 (0.03)
Median	0.91	0.89
25th, 75th Percentile	0.89, 0.93	0.87, 0.92
Min, Max	0.9, 0.9	0.9, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	4	8
Mean (SD)	0.00 (0.02)	0.00 (0.04)
Median	0.01	-0.01
25th, 75th Percentile	-0.02, 0.02	-0.03, 0.01
Min, Max	0.0, 0.0	0.0, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.02, 0.05)	0.00 (-0.03, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.2910

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.3

Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.75 (-2.05, 0.61)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
Baseline		
n	12	7
Mean (SD)	0.88 (0.03)	0.85 (0.07)
Median	0.87	0.86
25th, 75th Percentile	0.86, 0.90	0.81, 0.93
Min, Max	0.8, 0.9	0.7, 0.9
Week 52		
n	12	7
Mean (SD)	0.88 (0.03)	0.87 (0.05)
Median	0.87	0.86
25th, 75th Percentile	0.85, 0.90	0.83, 0.91
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.3
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	12	7
Mean (SD)	0.00 (0.02)	0.02 (0.08)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.01	-0.02, 0.00
Min, Max	-0.1, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.01, 0.03)	0.00 (-0.03, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.04, 0.03)
P-value ^b		0.7000

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.3
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by [Cohort 1]Age stratum for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

[Cohort 1]Age stratum Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.20 (-1.19, 0.80)
P-value for interaction term,treatment *[[Cohort 1]Age stratum]		0.8197

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and age stratum interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
<= 4.5		
Baseline		
n	11	6
Mean (SD)	0.88 (0.03)	0.87 (0.09)
Median	0.88	0.90
25th, 75th Percentile	0.85, 0.90	0.81, 0.93
Min, Max	0.8, 0.9	0.7, 0.9
Week 52		
n	11	6
Mean (SD)	0.88 (0.03)	0.89 (0.05)
Median	0.86	0.91
25th, 75th Percentile	0.85, 0.91	0.85, 0.92
Min, Max	0.8, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	11	6
Mean (SD)	0.00 (0.02)	0.02 (0.09)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.02	-0.02, 0.00
Min, Max	-0.1, 0.0	0.0, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.02)	0.02 (0.00, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.01, 0.05)
P-value ^b		0.2114

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.4
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
 Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		0.77 (-0.42, 1.92)

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
Baseline		
n	5	9
Mean (SD)	0.90 (0.03)	0.88 (0.03)
Median	0.90	0.88
25th, 75th Percentile	0.90, 0.91	0.86, 0.90
Min, Max	0.9, 0.9	0.8, 0.9
Week 52		
n	5	9
Mean (SD)	0.90 (0.02)	0.88 (0.03)
Median	0.90	0.87
25th, 75th Percentile	0.88, 0.90	0.87, 0.89
Min, Max	0.9, 0.9	0.8, 0.9

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 21JUN2023 06:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.006.102.004_mod_sub_agv_armsphgt_c1_206_fasr.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_206.sas, Database: N/A

Table 14.2.5.6.102.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
Change from baseline		
n	5	9
Mean (SD)	0.00 (0.02)	0.00 (0.04)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.00	-0.02, 0.00
Min, Max	0.0, 0.0	0.0, 0.1
LS mean change from baseline (95% CI)	0.02 (-0.02, 0.05)	-0.01 (-0.04, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.08, 0.02)
P-value ^b		0.2458

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.5.6.102.4
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)), by Baseline AGV for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

Baseline AGV Arm Span to Standing Height Ratio	Placebo (N=16)	Vosoritide (N=15)
SMD (95% CI) ^c		-0.85 (-2.22, 0.57)
P-value for interaction term,treatment * [Baseline AGV]		0.2443

^a Difference is vosoritide minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. Model terms included treatment, sex, age stratum, baseline age, baseline arm span to standing height ratio, and baseline AGV. For the overall analysis, which includes the interaction-term, model terms include sex, age stratum, baseline age, baseline AGV, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Table 14.2.15.1.101
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Height Z-Score at Week 52 (Overall) for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.2528
Ethnicity*Treatment Interaction	0.7009
Cohort 1 Age Stratum*Treatment Interaction	0.5259
Baseline AGV Category*Treatment Interaction	0.7164
Baseline Height Z-Score Category*Treatment Interaction	0.0776

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.001.101.000_mod_haz_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.1.102

Subgroup* Treatment Interaction P-values from Analysis of Covariance of Height Z-Score at Week 52 (Cohort 1 (≥ 24 to < 60 months)) for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex* Treatment Interaction	0.3017
Ethnicity* Treatment Interaction	0.8997
Cohort 1 Age Stratum* Treatment Interaction	0.5259
Baseline AGV Category* Treatment Interaction	0.3274

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.001.102.000_mod_haz_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.2.101
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Annualized Growth Velocity (AGV) at Week 52 (Overall) for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.8743
Ethnicity*Treatment Interaction	0.8446
Cohort 1 Age Stratum*Treatment Interaction	0.2855
Baseline AGV Category*Treatment Interaction	0.6683
Baseline Height Z-Score Category*Treatment Interaction	0.2061

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.002.101.000_mod_agv_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.2.102

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Annualized Growth Velocity (AGV) at Week 52 (Cohort 1 (≥ 24 to < 60 months)) for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.6994
Ethnicity*Treatment Interaction	0.8812
Cohort 1 Age Stratum*Treatment Interaction	0.2855
Baseline AGV Category*Treatment Interaction	0.1553

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.002.102.000_mod_agv_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.3.101
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Overall) for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.6026
Ethnicity*Treatment Interaction	0.6138
Cohort 1 Age Stratum*Treatment Interaction	0.5081
Baseline AGV Category*Treatment Interaction	0.8534
Baseline Height Z-Score Category*Treatment Interaction	0.8299

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.003.101.000_mod_bod_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.3.102

Subgroup* Treatment Interaction P-values from Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months))
for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex* Treatment Interaction	0.8809
Ethnicity* Treatment Interaction	0.6888
Cohort 1 Age Stratum* Treatment Interaction	0.5081
Baseline AGV Category* Treatment Interaction	0.8834

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.003.102.000_mod_bod_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.4.101
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Overall) for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5051
Ethnicity*Treatment Interaction	0.1767
Cohort 1 Age Stratum*Treatment Interaction	0.9959
Baseline AGV Category*Treatment Interaction	0.5483
Baseline Height Z-Score Category*Treatment Interaction	0.9510

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.004.101.000_mod_armrt_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.4.102

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 (Cohort 1 (>= 24 to < 60 months)) for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5800
Ethnicity*Treatment Interaction	0.5914
Cohort 1 Age Stratum*Treatment Interaction	0.9959
Baseline AGV Category*Treatment Interaction	0.2321

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.004.102.000_mod_armrt_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.5.101
Subgroup* Treatment Interaction P-values from Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Overall) for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex* Treatment Interaction	0.0495
Ethnicity* Treatment Interaction	0.4083
Cohort 1 Age Stratum* Treatment Interaction	0.4372
Baseline AGV Category* Treatment Interaction	0.2436
Baseline Height Z-Score Category* Treatment Interaction	0.5178

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.005.101.000_mod_legrt_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.5.102

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)) for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.0024
Ethnicity*Treatment Interaction	0.7559
Cohort 1 Age Stratum*Treatment Interaction	0.4372
Baseline AGV Category*Treatment Interaction	0.2399

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.005.102.000_mod_legrt_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.6.101
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Overall) for
BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.0074
Ethnicity*Treatment Interaction	0.6993
Cohort 1 Age Stratum*Treatment Interaction	0.7515
Baseline AGV Category*Treatment Interaction	0.1345
Baseline Height Z-Score Category*Treatment Interaction	0.0557

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.006.101.000_mod_legtrt_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.6.102

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)) for BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.0008
Ethnicity*Treatment Interaction	0.5923
Cohort 1 Age Stratum*Treatment Interaction	0.7515
Baseline AGV Category*Treatment Interaction	0.5797

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.006.102.000_mod_legtrt_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.7.101
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Overall) for BMN111-206
Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5379
Ethnicity*Treatment Interaction	0.6712
Cohort 1 Age Stratum*Treatment Interaction	0.8197
Baseline AGV Category*Treatment Interaction	0.1979
Baseline Height Z-Score Category*Treatment Interaction	0.1850

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.007.101.000_mod_armsphgt_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.7.102

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 (Cohort 1 (≥ 24 to < 60 months)) for
BMN111-206

Analysis Population: Full Analysis Set (Randomized Subjects)

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.4371
Ethnicity*Treatment Interaction	0.3448
Cohort 1 Age Stratum*Treatment Interaction	0.8197
Baseline AGV Category*Treatment Interaction	0.2443

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 06:17 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.007.102.000_mod_armsphgt_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.8.102.9
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Infant Toddler Quality of Life (ITQoL)
at Week 52 for BMN111-206: Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.5274
Ethnicity*Treatment Interaction	0.4142
Cohort 1 Age Stratum*Treatment Interaction	0.2469
Baseline AGV Category*Treatment Interaction	0.1439

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.008.102.009_qs_ovr_per_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.8.102.10
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Infant Toddler Quality of Life (ITQoL)
at Week 52 for BMN111-206: Change in Health Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.0419
Ethnicity*Treatment Interaction	0.1101
Cohort 1 Age Stratum*Treatment Interaction	0.6952
Baseline AGV Category*Treatment Interaction	0.9673

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.008.102.010_qs_ovr_chg_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.8.102.11
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Infant Toddler Quality of Life (ITQoL)
at Week 52 for BMN111-206: Parental Impact Emotional Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.0645
Ethnicity*Treatment Interaction	0.4651
Cohort 1 Age Stratum*Treatment Interaction	0.1467
Baseline AGV Category*Treatment Interaction	0.6487

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.008.102.011_qs_ovr_pie_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.8.102.12
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Infant Toddler Quality of Life (ITQoL)
at Week 52 for BMN111-206: Parental Impact Time Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.1611
Ethnicity*Treatment Interaction	0.5798
Cohort 1 Age Stratum*Treatment Interaction	0.2778
Baseline AGV Category*Treatment Interaction	0.6116

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.008.102.012_qs_ovr_pit_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.8.102.13
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Infant Toddler Quality of Life (ITQoL)
at Week 52 for BMN111-206: Family Cohesion Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.0350
Ethnicity*Treatment Interaction	0.8513
Cohort 1 Age Stratum*Treatment Interaction	0.1387
Baseline AGV Category*Treatment Interaction	0.8680

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.008.102.013_qs_ovr_coh_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.101.1
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Self-Care Score (Overall)
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9185
Ethnicity*Treatment Interaction	0.5913
Cohort 1 Age Stratum*Treatment Interaction	0.2262
Baseline AGV Category*Treatment Interaction	0.4646
Baseline Height Z-Score Category*Treatment Interaction	0.8947

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.101.001_qs_ovr_sel_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.101.2
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Mobility Score (Overall)
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.5846
Ethnicity*Treatment Interaction	0.4873
Cohort 1 Age Stratum*Treatment Interaction	0.2825
Baseline AGV Category*Treatment Interaction	0.0397
Baseline Height Z-Score Category*Treatment Interaction	0.6045

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.101.002_qs_ovr_mob_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.101.3
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Cognitive Score (Overall)
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.6969
Ethnicity*Treatment Interaction	0.7138
Cohort 1 Age Stratum*Treatment Interaction	0.9540
Baseline AGV Category*Treatment Interaction	0.2604
Baseline Height Z-Score Category*Treatment Interaction	0.8645

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.101.003_qs_ovr_cog_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.101.4
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Total Score (Overall)
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9245
Ethnicity*Treatment Interaction	0.5328
Cohort 1 Age Stratum*Treatment Interaction	0.3476
Baseline AGV Category*Treatment Interaction	0.1275
Baseline Height Z-Score Category*Treatment Interaction	0.7504

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.101.004_qs_ovr_tot_ov_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.102.1
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Self-Care Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9527
Ethnicity*Treatment Interaction	0.7081
Cohort 1 Age Stratum*Treatment Interaction	0.2262
Baseline AGV Category*Treatment Interaction	0.2207

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.102.001_qs_ovr_sel_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.102.2
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Mobility Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7049
Ethnicity*Treatment Interaction	0.7069
Cohort 1 Age Stratum*Treatment Interaction	0.2825
Baseline AGV Category*Treatment Interaction	0.1295

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.102.002_qs_ovr_mob_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.102.3
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Cognitive Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9688
Ethnicity*Treatment Interaction	0.4525
Cohort 1 Age Stratum*Treatment Interaction	0.9540
Baseline AGV Category*Treatment Interaction	0.3580

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.102.003_qs_ovr_cog_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.15.9.102.4
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure (WeeFIM)
at Week 52 for BMN111-206: Total Score (Cohort 1 (≥ 24 to < 60 months))
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.8730
Ethnicity*Treatment Interaction	0.8283
Cohort 1 Age Stratum*Treatment Interaction	0.3476
Baseline AGV Category*Treatment Interaction	0.1809

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 09:21 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.15.009.102.004_qs_ovr_tot_c1_int_pval_sub_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_206.sas, Database: N/A

Table 14.2.13.1.2.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Overall Health Score		
Baseline		
n	11	17
Mean (SD)	86.4 (17.9)	81.2 (15.4)
Median	100.0	85.0
25th, 75th Percentile	60.0, 100.0	60.0, 85.0
Min, Max	60, 100	60, 100
Week 26		
n	12	15
Mean (SD)	85.8 (14.0)	83.3 (21.4)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.002.101_qs_sum_ovr_qol_hlth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	10	15
Mean (SD)	-2.0 (16.7)	2.7 (18.1)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 15.0
Min, Max	-25, 25	-30, 25
Week 52		
n	13	12
Mean (SD)	88.8 (11.4)	86.3 (17.1)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.002.101_qs_sum_ovr_qol_hlth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	72.5, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	11	12
Mean (SD)	0.5 (17.8)	1.7 (16.4)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 25.0	-7.5, 15.0
Min, Max	-25, 25	-25, 25
Difference in change from baseline (95%CI)		1.21 (-13.63, 16.05)
P-value ^b		0.8668
Hedges'g (95% CI) ^c		0.07 (-0.75, 0.89)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.002.101_qs_sum_ovr_qol_hlth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Overall Health Score		
Baseline		
n	16	15
Mean (SD)	89.1 (10.7)	88.0 (15.9)
Median	85.0	100.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	16	14
Mean (SD)	91.9 (11.2)	87.9 (20.3)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.002.101_qs_sum_ovr_qol_hlth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	13	14
Mean (SD)	2.3 (18.3)	0.7 (16.2)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 0.0
Min, Max	-40, 40	-30, 40
Week 52		
n	15	15
Mean (SD)	86.7 (12.9)	89.7 (13.9)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.002.101_qs_sum_ovr_qol_hlth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	12	15
Mean (SD)	-1.3 (13.2)	1.7 (14.7)
Median	0.0	0.0
25th, 75th Percentile	-7.5, 0.0	0.0, 0.0
Min, Max	-25, 25	-25, 40
Difference in change from baseline (95%CI)		2.92 (-8.30, 14.13)
P-value ^b		0.5969
Hedges'g (95% CI) ^c		0.20 (-0.56, 0.96)
P-value for interaction term, treatment * [Sex]		0.8479

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.002.101_qs_sum_ovr_qol_hlth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Overall Health Score		
Baseline		
n	6	7
Mean (SD)	80.0 (21.9)	78.6 (18.4)
Median	80.0	85.0
25th, 75th Percentile	60.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	7	7
Mean (SD)	85.7 (13.4)	85.7 (13.4)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	6	7
Mean (SD)	3.3 (18.1)	7.1 (18.9)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 25.0	0.0, 25.0
Min, Max	-15, 25	-25, 25
Week 52		
n	7	4
Mean (SD)	89.3 (7.3)	76.3 (19.7)
Median	85.0	72.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.2.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	60.0, 92.5
Min, Max	85, 100	60, 100
Change from baseline to Week 52 ^a		
n	6	4
Mean (SD)	7.5 (19.9)	0.0 (20.4)
Median	12.5	0.0
25th, 75th Percentile	-15.0, 25.0	-12.5, 12.5
Min, Max	-15, 25	-25, 25
Difference in change from baseline (95%CI)		-7.50 (-37.44, 22.44)
P-value ^b		0.5794
Hedges'g (95% CI) ^c		-0.34 (-1.60, 0.95)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.2.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Overall Health Score		
Baseline		
n	8	8
Mean (SD)	83.8 (10.9)	86.3 (17.5)
Median	85.0	92.5
25th, 75th Percentile	85.0, 85.0	72.5, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	7	7
Mean (SD)	90.0 (15.0)	80.0 (26.3)
Median	100.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.2.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	6	7
Mean (SD)	5.0 (26.5)	-4.3 (14.3)
Median	7.5	0.0
25th, 75th Percentile	0.0, 15.0	-15.0, 0.0
Min, Max	-40, 40	-30, 15
Week 52		
n	7	8
Mean (SD)	85.7 (13.4)	94.4 (7.8)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.2.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 52 ^a		
n	6	8
Mean (SD)	0.0 (15.8)	8.1 (15.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 12.5
Min, Max	-25, 25	0, 40
Difference in change from baseline (95%CI)		8.13 (-10.31, 26.56)
P-value ^b		0.3560
Hedges'g (95% CI) ^c		0.49 (-0.60, 1.55)
P-value for interaction term, treatment * [Sex]		0.3037

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Overall Health Score		
Baseline		
n	22	21
Mean (SD)	87.0 (13.1)	82.9 (16.2)
Median	85.0	85.0
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	22	20
Mean (SD)	89.5 (12.0)	83.3 (23.1)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.3.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	72.5, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	19	20
Mean (SD)	2.1 (15.1)	0.5 (17.1)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 7.5
Min, Max	-25, 40	-30, 40
Week 52		
n	21	18
Mean (SD)	86.2 (10.8)	89.7 (13.0)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 85.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-1.9 (16.1)	4.4 (15.8)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 15.0
Min, Max	-25, 25	-25, 40
Difference in change from baseline (95%CI)		6.39 (-4.42, 17.19)
P-value ^b		0.2378
Hedges'g (95% CI) ^c		0.39 (-0.27, 1.05)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Overall Health Score		
Baseline		
n	5	11
Mean (SD)	92.0 (17.9)	87.3 (15.2)
Median	100.0	85.0
25th, 75th Percentile	100.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	6	9
Mean (SD)	88.3 (15.7)	90.6 (13.6)
Median	92.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	-7.5 (27.2)	4.4 (17.2)
Median	-7.5	0.0
25th, 75th Percentile	-27.5, 12.5	0.0, 15.0
Min, Max	-40, 25	-25, 25
Week 52		
n	7	9
Mean (SD)	92.1 (15.2)	85.0 (19.4)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.3.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	5	9
Mean (SD)	5.0 (11.2)	-3.9 (12.9)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	0, 25	-25, 15
Difference in change from baseline (95%CI)		-8.89 (-23.93, 6.16)
P-value ^b		0.2222
Hedges'g (95% CI) ^c		-0.67 (-1.78, 0.47)
P-value for interaction term, treatment * [Ethnicity]		0.1263

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Overall Health Score		
Baseline		
n	12	8
Mean (SD)	82.5 (15.0)	79.4 (17.2)
Median	85.0	85.0
25th, 75th Percentile	72.5, 92.5	60.0, 92.5
Min, Max	60, 100	60, 100
Week 26		
n	11	8
Mean (SD)	89.5 (12.3)	75.6 (24.0)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	60.0, 92.5
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	6.5 (17.3)	-3.8 (17.1)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	-12.5, 0.0
Min, Max	-15, 40	-30, 25
Week 52		
n	11	7
Mean (SD)	85.5 (10.4)	87.9 (14.4)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.1.3.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 85.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	10	7
Mean (SD)	2.0 (18.0)	9.3 (21.9)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 25.0	0.0, 25.0
Min, Max	-25, 25	-25, 40
Difference in change from baseline (95%CI)		7.29 (-13.34, 27.91)
P-value ^b		0.4631
Hedges'g (95% CI) ^c		0.35 (-0.63, 1.32)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.003.102_qs_sum_ovr_qol_hlth_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.3.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Overall Health Score		
Baseline		
n	2	7
Mean (SD)	80.0 (28.3)	86.4 (18.9)
Median	80.0	100.0
25th, 75th Percentile	60.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	3	6
Mean (SD)	81.7 (20.2)	92.5 (8.2)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.3.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 26 ^a		
n	2	6
Mean (SD)	-7.5 (46.0)	8.3 (16.0)
Median	-7.5	7.5
25th, 75th Percentile	-40.0, 25.0	0.0, 25.0
Min, Max	-40, 25	-15, 25
Week 52		
n	3	5
Mean (SD)	95.0 (8.7)	89.0 (17.5)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.003.102_qs_sum_ovr_qol_hlth_eth_c1_206_fas.pdf+rtf

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Table 14.2.13.1.3.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	60, 100
Change from baseline to Week 52 ^a		
n	2	5
Mean (SD)	12.5 (17.7)	0.0 (0.0)
Median	12.5	0.0
25th, 75th Percentile	0.0, 25.0	0.0, 0.0
Min, Max	0, 25	0, 0
Difference in change from baseline (95%CI)		-12.50 (-171.33, 146.33)
P-value ^b		0.5000
Hedges'g (95% CI) ^c		-1.33 (-3.11, 0.55)
P-value for interaction term, treatment * [Ethnicity]		0.2568

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.4.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Overall Health Score		
Baseline		
n	3	8
Mean (SD)	86.7 (23.1)	83.1 (19.8)
Median	100.0	92.5
25th, 75th Percentile	60.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	4	7
Mean (SD)	88.8 (7.5)	81.4 (23.8)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 92.5	85.0, 100.0
Min, Max	85, 100	30, 100
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	-1.7 (23.1)	0.7 (19.9)
Median	-15.0	0.0
25th, 75th Percentile	-15.0, 25.0	-15.0, 25.0
Min, Max	-15, 25	-30, 25
Week 52		
n	4	6
Mean (SD)	88.8 (7.5)	90.8 (16.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 92.5	85.0, 100.0
Min, Max	85, 100	60, 100
Change from baseline to Week 52 ^a		
n	3	6
Mean (SD)	-1.7 (23.1)	6.7 (16.3)
Median	-15.0	0.0
25th, 75th Percentile	-15.0, 25.0	0.0, 0.0
Min, Max	-15, 25	0, 40
Difference in change from baseline (95%CI)		8.33 (-22.63, 39.29)
P-value ^b		0.5447
Hedges'g (95% CI) ^c		0.40 (-1.01, 1.79)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Overall Health Score		
Baseline		
n	11	7
Mean (SD)	80.9 (14.6)	82.1 (16.5)
Median	85.0	85.0
25th, 75th Percentile	60.0, 85.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	10	7
Mean (SD)	87.5 (16.0)	84.3 (17.9)
Median	92.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	6.1 (22.2)	2.1 (15.5)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 15.0
Min, Max	-40, 40	-25, 25
Week 52		
n	10	6
Mean (SD)	87.0 (11.8)	85.8 (14.6)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	9	6
Mean (SD)	5.6 (16.7)	4.2 (18.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 25.0	0.0, 25.0
Min, Max	-25, 25	-25, 25
Difference in change from baseline (95%CI)		-1.39 (-21.34, 18.57)
P-value ^b		0.8828
Hedges'g (95% CI) ^c		-0.07 (-1.11, 0.96)
P-value for interaction term, treatment *[Cohort 1 Age Stratum]		0.5445

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Overall Health Score		
Baseline		
n	3	8
Mean (SD)	86.7 (23.1)	83.1 (19.8)
Median	100.0	92.5
25th, 75th Percentile	60.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	4	7
Mean (SD)	88.8 (7.5)	81.4 (23.8)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 92.5	85.0, 100.0
Min, Max	85, 100	30, 100
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	-1.7 (23.1)	0.7 (19.9)
Median	-15.0	0.0
25th, 75th Percentile	-15.0, 25.0	-15.0, 25.0
Min, Max	-15, 25	-30, 25
Week 52		
n	4	6
Mean (SD)	88.8 (7.5)	90.8 (16.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 92.5	85.0, 100.0
Min, Max	85, 100	60, 100
Change from baseline to Week 52 ^a		
n	3	6
Mean (SD)	-1.7 (23.1)	6.7 (16.3)
Median	-15.0	0.0
25th, 75th Percentile	-15.0, 25.0	0.0, 0.0
Min, Max	-15, 25	0, 40
Difference in change from baseline (95%CI)		8.33 (-22.63, 39.29)
P-value ^b		0.5447
Hedges'g (95% CI) ^c		0.40 (-1.01, 1.79)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Overall Health Score		
Baseline		
n	11	7
Mean (SD)	80.9 (14.6)	82.1 (16.5)
Median	85.0	85.0
25th, 75th Percentile	60.0, 85.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	10	7
Mean (SD)	87.5 (16.0)	84.3 (17.9)
Median	92.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	6.1 (22.2)	2.1 (15.5)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 15.0
Min, Max	-40, 40	-25, 25
Week 52		
n	10	6
Mean (SD)	87.0 (11.8)	85.8 (14.6)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.1.4.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	9	6
Mean (SD)	5.6 (16.7)	4.2 (18.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 25.0	0.0, 25.0
Min, Max	-25, 25	-25, 25
Difference in change from baseline (95%CI)		-1.39 (-21.34, 18.57)
P-value ^b		0.8828
Hedges'g (95% CI) ^c		-0.07 (-1.11, 0.96)
P-value for interaction term, treatment *[Cohort 1 Age Stratum]		0.5445

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.004.102_qs_sum_ovr_qol_hlth_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Overall Health Score		
Baseline		
n	10	7
Mean (SD)	84.5 (14.6)	87.9 (14.4)
Median	85.0	85.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	11	7
Mean (SD)	92.3 (12.7)	90.0 (15.0)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.005.101_qs_sum_ovr_qol_hlth_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	6.1 (15.6)	2.1 (15.5)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 15.0
Min, Max	-15, 40	-25, 25
Week 52		
n	11	5
Mean (SD)	86.8 (11.2)	86.0 (16.4)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	9	5
Mean (SD)	-0.6 (17.0)	0.0 (17.7)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 0.0
Min, Max	-25, 25	-25, 25
Difference in change from baseline (95%CI)		0.56 (-20.41, 21.52)
P-value ^b		0.9549
Hedges'g (95% CI) ^c		0.03 (-1.06, 1.12)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.005.101_qs_sum_ovr_qol_hlth_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Overall Health Score		
Baseline		
n	17	25
Mean (SD)	90.0 (13.3)	83.4 (16.2)
Median	100.0	85.0
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	17	22
Mean (SD)	87.4 (12.5)	84.1 (22.2)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.005.101_qs_sum_ovr_qol_hlth_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	14	22
Mean (SD)	-3.2 (18.0)	1.6 (17.7)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 15.0
Min, Max	-40, 25	-30, 40
Week 52		
n	17	22
Mean (SD)	88.2 (12.9)	88.6 (15.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	14	22
Mean (SD)	-0.4 (14.6)	2.0 (15.0)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 15.0
Min, Max	-25, 25	-25, 40
Difference in change from baseline (95%CI)		2.40 (-7.92, 12.73)
P-value ^b		0.6393
Hedges'g (95% CI) ^c		0.16 (-0.51, 0.83)
P-value for interaction term, treatment * [Baseline AGV Category]		0.8564

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
<= 4.5		
ITQoL : Overall Health Score		
Baseline		
n	9	6
Mean (SD)	82.8 (14.4)	88.3 (15.7)
Median	85.0	92.5
25th, 75th Percentile	85.0, 85.0	85.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	10	6
Mean (SD)	91.5 (13.1)	95.0 (7.7)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 26 ^a		
n	8	6
Mean (SD)	6.9 (16.5)	6.7 (10.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 15.0
Min, Max	-15, 40	0, 25
Week 52		
n	10	4
Mean (SD)	87.0 (11.8)	92.5 (8.7)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 52 ^a		
n	8	4
Mean (SD)	1.3 (17.3)	6.3 (12.5)
Median	0.0	0.0
25th, 75th Percentile	-7.5, 12.5	0.0, 12.5
Min, Max	-25, 25	0, 25
Difference in change from baseline (95%CI)		5.00 (-16.82, 26.82)
P-value ^b		0.6207
Hedges'g (95% CI) ^c		0.29 (-0.93, 1.49)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Overall Health Score		
Baseline		
n	5	9
Mean (SD)	81.0 (20.1)	78.9 (18.8)
Median	85.0	85.0
25th, 75th Percentile	60.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	4	8
Mean (SD)	78.8 (12.5)	73.8 (22.3)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.5, 85.0	60.0, 85.0
Min, Max	60, 85	30, 100
Change from baseline to Week 26 ^a		
n	4	8
Mean (SD)	-1.3 (32.0)	-2.5 (20.5)
Median	5.0	0.0
25th, 75th Percentile	-27.5, 25.0	-20.0, 12.5
Min, Max	-40, 25	-30, 25
Week 52		
n	4	8
Mean (SD)	88.8 (7.5)	86.3 (17.5)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.005.102_qs_sum_ovr_qol_hlth_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.5.102
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 92.5	72.5, 100.0
Min, Max	85, 100	60, 100
Change from baseline to Week 52 ^a		
n	4	8
Mean (SD)	8.8 (19.7)	5.0 (19.5)
Median	12.5	0.0
25th, 75th Percentile	-7.5, 25.0	0.0, 12.5
Min, Max	-15, 25	-25, 40
Difference in change from baseline (95%CI)		-3.75 (-30.41, 22.91)
P-value ^b		0.7604
Hedges'g (95% CI) ^c		-0.18 (-1.38, 1.03)
P-value for interaction term, treatment *[Baseline AGV Category]		0.5777

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.6.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Overall Health Score		
Baseline		
n	17	13
Mean (SD)	85.9 (14.2)	89.2 (14.7)
Median	85.0	100.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	15	11
Mean (SD)	87.0 (15.6)	90.9 (12.6)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.6.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	14	11
Mean (SD)	1.1 (20.7)	2.3 (15.1)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 15.0	0.0, 15.0
Min, Max	-40, 40	-25, 25
Week 52		
n	15	11
Mean (SD)	86.7 (12.9)	90.0 (16.0)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.6.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	14	11
Mean (SD)	0.7 (17.1)	1.4 (12.1)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 15.0	0.0, 0.0
Min, Max	-25, 25	-25, 25
Difference in change from baseline (95%CI)		0.65 (-11.94, 13.24)
P-value ^b		0.9160
Hedges'g (95% CI) ^c		0.04 (-0.75, 0.83)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.001.006.101_qs_sum_ovr_qol_hlth_haz_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.6.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Overall Health Score		
Baseline		
n	10	19
Mean (SD)	91.5 (13.1)	81.1 (15.9)
Median	100.0	85.0
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	13	18
Mean (SD)	91.9 (7.8)	82.2 (24.0)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.6.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	85, 100	30, 100
Change from baseline to Week 26 ^a		
n	9	18
Mean (SD)	-0.6 (11.6)	1.4 (18.4)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 15.0
Min, Max	-15, 25	-30, 40
Week 52		
n	13	16
Mean (SD)	88.8 (11.4)	86.9 (15.0)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.1.6.101
Infant Toddler Quality of Life (ITQoL): Overall Health Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	9	16
Mean (SD)	-2.2 (12.5)	1.9 (17.4)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	-7.5, 15.0
Min, Max	-15, 25	-25, 40
Difference in change from baseline (95%CI)		4.10 (-9.59, 17.78)
P-value ^b		0.5418
Hedges'g (95% CI) ^c		0.25 (-0.57, 1.07)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.7028

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Physical Abilities Score		
Baseline		
n	13	16
Mean (SD)	79.2 (15.9)	71.8 (28.5)
Median	85.2	79.8
25th, 75th Percentile	76.7, 90.0	61.7, 93.0
Min, Max	37, 93	0, 100
Week 26		
n	12	16
Mean (SD)	77.5 (18.0)	74.6 (19.8)
Median	83.3	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.7, 89.7	58.3, 91.1
Min, Max	47, 100	40, 100
Change from baseline to Week 26 ^a		
n	12	15
Mean (SD)	-0.6 (15.2)	1.4 (33.7)
Median	-1.7	-9.3
25th, 75th Percentile	-5.9, 10.4	-16.7, 6.7
Min, Max	-30, 24	-43, 80
Week 52		
n	13	15
Mean (SD)	79.6 (17.0)	79.3 (16.1)
Median	81.5	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.2.2.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	73.3, 90.0	73.3, 90.0
Min, Max	40, 100	43, 100
Change from baseline to Week 52 ^a		
n	13	14
Mean (SD)	0.4 (10.5)	2.9 (25.7)
Median	3.3	2.8
25th, 75th Percentile	-3.3, 4.8	-12.2, 7.4
Min, Max	-27, 13	-37, 73
Difference in change from baseline (95%CI)		2.58 (-13.12, 18.27)
P-value ^b		0.7339
Hedges'g (95% CI) ^c		0.13 (-0.63, 0.88)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Physical Abilities Score		
Baseline		
n	17	13
Mean (SD)	74.4 (25.3)	77.4 (21.3)
Median	80.0	76.7
25th, 75th Percentile	66.7, 94.4	70.0, 94.4
Min, Max	11, 100	33, 100
Week 26		
n	16	15
Mean (SD)	77.1 (24.8)	87.2 (11.3)
Median	84.3	90.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	68.3, 95.0	80.0, 96.3
Min, Max	13, 100	60, 100
Change from baseline to Week 26 ^a		
n	14	13
Mean (SD)	0.5 (16.6)	8.7 (21.1)
Median	3.3	0.0
25th, 75th Percentile	-8.5, 6.7	-3.3, 10.0
Min, Max	-28, 38	-10, 60
Week 52		
n	17	15
Mean (SD)	78.7 (21.3)	77.9 (18.0)
Median	83.3	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	74.1, 96.3	70.0, 90.0
Min, Max	20, 100	41, 100
Change from baseline to Week 52 ^a		
n	15	13
Mean (SD)	-3.3 (20.7)	1.5 (21.4)
Median	-6.7	3.3
25th, 75th Percentile	-11.1, 0.0	0.0, 9.6
Min, Max	-44, 49	-54, 40
Difference in change from baseline (95%CI)		4.76 (-11.61, 21.13)
P-value ^b		0.5554
Hedges'g (95% CI) ^c		0.22 (-0.53, 0.96)
P-value for interaction term, treatment * [Sex]		0.8443

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Physical Abilities Score		
Baseline		
n	7	7
Mean (SD)	78.4 (18.9)	64.1 (39.1)
Median	85.2	83.3
25th, 75th Percentile	76.7, 90.0	20.0, 93.3
Min, Max	37, 90	0, 100
Week 26		
n	7	7
Mean (SD)	71.9 (19.9)	82.4 (10.8)
Median	80.0	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	50.0, 83.3	73.3, 93.3
Min, Max	47, 100	70, 100
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	-6.5 (15.9)	18.3 (41.9)
Median	-5.2	0.0
25th, 75th Percentile	-23.3, 10.0	-10.0, 76.7
Min, Max	-30, 13	-20, 80
Week 52		
n	7	6
Mean (SD)	76.2 (21.1)	76.7 (17.8)
Median	86.7	81.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.2.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	56.7, 90.0	73.3, 90.0
Min, Max	40, 100	43, 90
Change from baseline to Week 52 ^a		
n	7	6
Mean (SD)	-2.2 (11.8)	5.2 (34.5)
Median	0.0	-8.3
25th, 75th Percentile	-3.3, 4.8	-12.2, 6.7
Min, Max	-27, 10	-20, 73
Difference in change from baseline (95%CI)		7.36 (-28.80, 43.51)
P-value ^b		0.6363
Hedges'g (95% CI) ^c		0.28 (-0.83, 1.37)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.2.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Physical Abilities Score		
Baseline		
n	8	8
Mean (SD)	77.5 (24.1)	72.2 (24.6)
Median	83.3	73.3
25th, 75th Percentile	68.3, 96.7	53.9, 95.0
Min, Max	27, 97	33, 100
Week 26		
n	7	8
Mean (SD)	83.5 (23.8)	85.8 (11.9)
Median	90.0	88.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.2.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 100.0	83.3, 91.7
Min, Max	33, 100	60, 100
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	0.8 (6.7)	13.6 (26.1)
Median	3.3	5.0
25th, 75th Percentile	-6.7, 6.7	-6.7, 31.1
Min, Max	-9, 7	-10, 60
Week 52		
n	8	8
Mean (SD)	73.5 (26.5)	81.3 (16.8)
Median	83.3	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.2.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	58.9, 91.7	71.7, 95.0
Min, Max	20, 100	50, 100
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	-9.8 (6.0)	9.0 (14.4)
Median	-10.0	4.4
25th, 75th Percentile	-12.2, -6.7	0.0, 13.3
Min, Max	-20, 0	-3, 40
Difference in change from baseline (95%CI)		18.87 (6.41, 31.33)
P-value ^b		0.0072
Hedges'g (95% CI) ^c		1.57 (0.37, 2.73)
P-value for interaction term, treatment * [Sex]		0.4273

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.002.002.102_qs_sum_ovr_qol_phys_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Physical Abilities Score		
Baseline		
n	24	20
Mean (SD)	75.9 (22.8)	74.1 (22.4)
Median	83.3	74.2
25th, 75th Percentile	66.7, 90.0	63.3, 94.4
Min, Max	11, 100	20, 100
Week 26		
n	22	20
Mean (SD)	80.5 (17.5)	79.2 (18.5)
Median	83.3	84.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.002.003.101_qs_sum_ovr_qol_phys_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.2.3.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 90.5	68.3, 94.8
Min, Max	33, 100	40, 100
Change from baseline to Week 26 ^a		
n	21	19
Mean (SD)	0.1 (12.5)	4.2 (25.2)
Median	0.0	0.0
25th, 75th Percentile	-6.7, 6.7	-10.0, 6.7
Min, Max	-28, 24	-23, 80
Week 52		
n	23	19
Mean (SD)	77.9 (20.5)	79.7 (16.9)
Median	83.3	85.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 92.6	70.0, 90.0
Min, Max	20, 100	41, 100
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	-2.5 (14.0)	2.2 (20.3)
Median	-0.7	6.7
25th, 75th Percentile	-10.0, 4.8	-1.1, 10.2
Min, Max	-44, 26	-54, 40
Difference in change from baseline (95%CI)		4.69 (-6.31, 15.68)
P-value ^b		0.3938
Hedges'g (95% CI) ^c		0.27 (-0.36, 0.89)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.002.003.101_qs_sum_ovr_qol_phys_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Physical Abilities Score		
Baseline		
n	6	9
Mean (SD)	79.0 (17.5)	74.7 (32.4)
Median	80.0	88.9
25th, 75th Percentile	76.7, 93.3	76.7, 92.6
Min, Max	48, 97	0, 100
Week 26		
n	6	11
Mean (SD)	65.3 (32.4)	83.5 (15.1)
Median	72.6	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	46.7, 87.5	80.0, 93.3
Min, Max	13, 100	43, 100
Change from baseline to Week 26 ^a		
n	5	9
Mean (SD)	-0.3 (27.3)	6.0 (35.6)
Median	3.3	0.0
25th, 75th Percentile	-23.3, 10.8	-10.0, 10.0
Min, Max	-30, 38	-43, 77
Week 52		
n	7	11
Mean (SD)	83.0 (14.9)	76.6 (17.4)
Median	81.5	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	73.3, 96.3	56.7, 90.0
Min, Max	57, 97	50, 100
Change from baseline to Week 52 ^a		
n	6	9
Mean (SD)	1.7 (25.4)	2.3 (29.7)
Median	-1.7	-3.3
25th, 75th Percentile	-11.9, 3.3	-10.0, 5.6
Min, Max	-27, 49	-37, 73
Difference in change from baseline (95%CI)		0.61 (-31.44, 32.66)
P-value ^b		0.9677
Hedges'g (95% CI) ^c		0.02 (-1.01, 1.05)
P-value for interaction term, treatment * [Ethnicity]		0.7480

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Physical Abilities Score		
Baseline		
n	12	8
Mean (SD)	76.0 (22.9)	66.7 (28.8)
Median	85.9	66.7
25th, 75th Percentile	68.3, 90.0	48.3, 91.7
Min, Max	27, 97	20, 100
Week 26		
n	11	8
Mean (SD)	80.1 (21.0)	84.2 (14.8)
Median	83.3	88.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 100.0	71.7, 96.7
Min, Max	33, 100	60, 100
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	1.0 (7.9)	17.5 (34.0)
Median	0.0	3.3
25th, 75th Percentile	-6.7, 6.7	-6.7, 38.3
Min, Max	-9, 13	-10, 80
Week 52		
n	12	7
Mean (SD)	74.5 (24.9)	81.0 (20.3)
Median	86.7	90.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	58.9, 88.3	70.0, 100.0
Min, Max	20, 100	43, 100
Change from baseline to Week 52 ^a		
n	11	7
Mean (SD)	-4.9 (8.8)	7.6 (18.6)
Median	-6.7	6.7
25th, 75th Percentile	-10.0, 3.3	0.0, 20.0
Min, Max	-20, 10	-20, 40
Difference in change from baseline (95%CI)		12.54 (-4.92, 29.99)
P-value ^b		0.1356
Hedges'g (95% CI) ^c		0.89 (-0.12, 1.88)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Physical Abilities Score		
Baseline		
n	3	7
Mean (SD)	85.6 (10.2)	70.5 (36.0)
Median	83.3	88.9
25th, 75th Percentile	76.7, 96.7	44.4, 93.3
Min, Max	77, 97	0, 100
Week 26		
n	3	7
Mean (SD)	68.9 (27.8)	84.3 (6.0)
Median	60.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.3.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	46.7, 100.0	80.0, 90.0
Min, Max	47, 100	77, 93
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	-16.7 (17.6)	13.8 (34.8)
Median	-23.3	0.0
25th, 75th Percentile	-30.0, 3.3	-10.0, 45.6
Min, Max	-30, 3	-20, 77
Week 52		
n	3	7
Mean (SD)	75.6 (20.1)	77.6 (13.6)
Median	73.3	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.3.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	56.7, 96.7	73.3, 86.7
Min, Max	57, 97	50, 90
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	-10.0 (14.5)	7.1 (29.9)
Median	-3.3	-3.3
25th, 75th Percentile	-26.7, 0.0	-10.0, 5.6
Min, Max	-27, 0	-12, 73
Difference in change from baseline (95%CI)		17.14 (-25.66, 59.95)
P-value ^b		0.3827
Hedges'g (95% CI) ^c		0.58 (-0.82, 1.94)
P-value for interaction term, treatment * [Ethnicity]		0.7757

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.4.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Physical Abilities Score		
Baseline		
n	3	8
Mean (SD)	84.0 (6.8)	63.3 (34.6)
Median	85.2	73.3
25th, 75th Percentile	76.7, 90.0	38.9, 91.1
Min, Max	77, 90	0, 100
Week 26		
n	4	8
Mean (SD)	77.5 (22.3)	86.3 (6.0)
Median	81.7	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.4.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	63.3, 91.7	81.7, 91.7
Min, Max	47, 100	77, 93
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	-14.0 (13.9)	22.9 (34.1)
Median	-6.7	13.3
25th, 75th Percentile	-30.0, -5.2	-2.8, 52.8
Min, Max	-30, -5	-20, 77
Week 52		
n	4	8
Mean (SD)	87.5 (11.0)	77.5 (13.1)
Median	88.3	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.4.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 95.0	73.3, 88.3
Min, Max	73, 100	50, 90
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	-0.6 (4.7)	14.2 (29.5)
Median	-3.3	4.4
25th, 75th Percentile	-3.3, 4.8	-8.3, 30.0
Min, Max	-3, 5	-12, 73
Difference in change from baseline (95%CI)		14.79 (-10.18, 39.75)
P-value ^b		0.2084
Hedges'g (95% CI) ^c		0.52 (-0.84, 1.85)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.002.004.101_qs_sum_ovr_qol_phys_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.4.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Physical Abilities Score		
Baseline		
n	12	7
Mean (SD)	76.4 (23.3)	74.3 (28.4)
Median	85.0	83.3
25th, 75th Percentile	68.3, 93.3	63.3, 100.0
Min, Max	27, 97	20, 100
Week 26		
n	10	7
Mean (SD)	77.8 (23.0)	81.9 (15.4)
Median	82.4	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.4.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 100.0	70.0, 100.0
Min, Max	33, 100	60, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.1 (10.9)	7.6 (32.5)
Median	3.3	-3.3
25th, 75th Percentile	-6.7, 6.7	-10.0, 6.7
Min, Max	-23, 13	-10, 80
Week 52		
n	11	6
Mean (SD)	70.1 (25.3)	81.7 (21.8)
Median	80.0	88.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.4.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	56.7, 86.7	70.0, 100.0
Min, Max	20, 100	43, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-7.5 (10.5)	-1.7 (9.8)
Median	-10.0	0.0
25th, 75th Percentile	-12.2, 0.0	-3.3, 6.7
Min, Max	-27, 10	-20, 7
Difference in change from baseline (95%CI)		5.81 (-5.31, 16.93)
P-value ^b		0.2829
Hedges'g (95% CI) ^c		0.54 (-0.48, 1.54)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.5600

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.4.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Physical Abilities Score		
Baseline		
n	3	8
Mean (SD)	84.0 (6.8)	63.3 (34.6)
Median	85.2	73.3
25th, 75th Percentile	76.7, 90.0	38.9, 91.1
Min, Max	77, 90	0, 100
Week 26		
n	4	8
Mean (SD)	77.5 (22.3)	86.3 (6.0)
Median	81.7	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.4.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	63.3, 91.7	81.7, 91.7
Min, Max	47, 100	77, 93
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	-14.0 (13.9)	22.9 (34.1)
Median	-6.7	13.3
25th, 75th Percentile	-30.0, -5.2	-2.8, 52.8
Min, Max	-30, -5	-20, 77
Week 52		
n	4	8
Mean (SD)	87.5 (11.0)	77.5 (13.1)
Median	88.3	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.4.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 95.0	73.3, 88.3
Min, Max	73, 100	50, 90
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	-0.6 (4.7)	14.2 (29.5)
Median	-3.3	4.4
25th, 75th Percentile	-3.3, 4.8	-8.3, 30.0
Min, Max	-3, 5	-12, 73
Difference in change from baseline (95%CI)		14.79 (-10.18, 39.75)
P-value ^b		0.2084
Hedges'g (95% CI) ^c		0.52 (-0.84, 1.85)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.4.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Physical Abilities Score		
Baseline		
n	12	7
Mean (SD)	76.4 (23.3)	74.3 (28.4)
Median	85.0	83.3
25th, 75th Percentile	68.3, 93.3	63.3, 100.0
Min, Max	27, 97	20, 100
Week 26		
n	10	7
Mean (SD)	77.8 (23.0)	81.9 (15.4)
Median	82.4	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.4.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 100.0	70.0, 100.0
Min, Max	33, 100	60, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.1 (10.9)	7.6 (32.5)
Median	3.3	-3.3
25th, 75th Percentile	-6.7, 6.7	-10.0, 6.7
Min, Max	-23, 13	-10, 80
Week 52		
n	11	6
Mean (SD)	70.1 (25.3)	81.7 (21.8)
Median	80.0	88.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

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Table 14.2.13.2.4.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	56.7, 86.7	70.0, 100.0
Min, Max	20, 100	43, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-7.5 (10.5)	-1.7 (9.8)
Median	-10.0	0.0
25th, 75th Percentile	-12.2, 0.0	-3.3, 6.7
Min, Max	-27, 10	-20, 7
Difference in change from baseline (95%CI)		5.81 (-5.31, 16.93)
P-value ^b		0.2829
Hedges'g (95% CI) ^c		0.54 (-0.48, 1.54)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.5600

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

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Table 14.2.13.2.5.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Physical Abilities Score		
Baseline		
n	11	7
Mean (SD)	77.1 (23.7)	65.7 (40.4)
Median	85.2	86.7
25th, 75th Percentile	73.3, 92.6	20.0, 100.0
Min, Max	27, 97	0, 100
Week 26		
n	11	7
Mean (SD)	78.5 (22.0)	78.6 (19.3)
Median	81.5	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.5.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 100.0	70.0, 100.0
Min, Max	33, 100	43, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-0.7 (10.9)	12.9 (47.5)
Median	-0.2	0.0
25th, 75th Percentile	-6.7, 6.7	-20.0, 76.7
Min, Max	-23, 13	-43, 80
Week 52		
n	12	6
Mean (SD)	72.6 (25.5)	73.9 (22.8)
Median	83.3	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.5.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	56.7, 91.7	50.0, 90.0
Min, Max	20, 100	43, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-7.0 (11.0)	0.6 (38.0)
Median	-10.0	-6.7
25th, 75th Percentile	-12.2, 3.3	-20.0, 0.0
Min, Max	-27, 10	-37, 73
Difference in change from baseline (95%CI)		7.53 (-32.27, 47.32)
P-value ^b		0.6538
Hedges'g (95% CI) ^c		0.30 (-0.70, 1.30)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.5.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Physical Abilities Score		
Baseline		
n	19	22
Mean (SD)	76.2 (20.9)	77.1 (18.8)
Median	80.0	76.4
25th, 75th Percentile	66.7, 90.0	66.7, 93.3
Min, Max	11, 100	33, 100
Week 26		
n	17	24
Mean (SD)	76.4 (22.3)	81.3 (17.0)
Median	83.3	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.5.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 87.5	71.9, 93.3
Min, Max	13, 100	40, 100
Change from baseline to Week 26 ^a		
n	16	21
Mean (SD)	0.5 (18.3)	2.1 (19.3)
Median	1.7	0.0
25th, 75th Percentile	-12.5, 9.1	-10.0, 5.6
Min, Max	-30, 38	-23, 60
Week 52		
n	18	24
Mean (SD)	83.4 (12.7)	79.7 (15.4)
Median	82.4	82.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.2.5.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	76.7, 96.3	71.7, 90.0
Min, Max	50, 100	41, 100
Change from baseline to Week 52 ^a		
n	17	21
Mean (SD)	1.9 (18.8)	2.7 (18.5)
Median	0.0	6.7
25th, 75th Percentile	-3.3, 8.3	-1.1, 9.6
Min, Max	-44, 49	-54, 40
Difference in change from baseline (95%CI)		0.82 (-11.51, 13.15)
P-value ^b		0.8940
Hedges'g (95% CI) ^c		0.04 (-0.60, 0.68)
P-value for interaction term, treatment * [Baseline AGV Category]		0.5855

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.002.005.101_qs_sum_ovr_qol_phys_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.5.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
ITQoL : Physical Abilities Score		
Baseline		
n	10	6
Mean (SD)	75.5 (24.4)	62.2 (43.1)
Median	84.3	76.7
25th, 75th Percentile	73.3, 90.0	20.0, 100.0
Min, Max	27, 97	0, 100
Week 26		
n	10	6
Mean (SD)	77.5 (22.9)	84.4 (12.6)
Median	80.7	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.5.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 100.0	76.7, 100.0
Min, Max	33, 100	70, 100
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	-0.4 (11.5)	22.2 (44.4)
Median	3.3	3.3
25th, 75th Percentile	-6.7, 6.7	-10.0, 76.7
Min, Max	-23, 13	-20, 80
Week 52		
n	11	5
Mean (SD)	70.7 (25.9)	78.7 (21.9)
Median	80.0	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.2.5.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	56.7, 90.0	73.3, 90.0
Min, Max	20, 100	43, 100
Change from baseline to Week 52 ^a		
n	10	5
Mean (SD)	-7.7 (11.3)	8.0 (37.3)
Median	-10.0	-3.3
25th, 75th Percentile	-12.2, 3.3	-10.0, 0.0
Min, Max	-27, 10	-20, 73
Difference in change from baseline (95%CI)		15.74 (-30.09, 61.58)
P-value ^b		0.4043
Hedges'g (95% CI) ^c		0.65 (-0.46, 1.74)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.5.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Physical Abilities Score		
Baseline		
n	5	9
Mean (SD)	82.7 (13.0)	72.6 (22.4)
Median	86.7	76.7
25th, 75th Percentile	76.7, 90.0	63.3, 88.9
Min, Max	63, 97	33, 100
Week 26		
n	4	9
Mean (SD)	78.3 (22.5)	84.1 (10.9)
Median	83.3	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.5.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	65.0, 91.7	83.3, 90.0
Min, Max	47, 100	60, 93
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	-9.2 (14.5)	11.5 (25.3)
Median	-5.0	0.0
25th, 75th Percentile	-18.3, 0.0	-5.6, 16.7
Min, Max	-30, 3	-10, 60
Week 52		
n	4	9
Mean (SD)	85.8 (9.6)	79.6 (14.6)
Median	86.7	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.5.102
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 91.7	73.3, 90.0
Min, Max	73, 97	50, 100
Change from baseline to Week 52 ^a		
n	4	9
Mean (SD)	-1.7 (1.9)	7.0 (15.3)
Median	-1.7	5.6
25th, 75th Percentile	-3.3, 0.0	0.0, 6.7
Min, Max	-3, 0	-12, 40
Difference in change from baseline (95%CI)		8.71 (-3.15, 20.56)
P-value ^b		0.1301
Hedges'g (95% CI) ^c		0.62 (-0.60, 1.81)
P-value for interaction term, treatment * [Baseline AGV Category]		0.6517

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.6.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Physical Abilities Score		
Baseline		
n	18	13
Mean (SD)	73.4 (25.0)	72.0 (32.0)
Median	79.1	83.3
25th, 75th Percentile	63.3, 90.0	63.3, 92.6
Min, Max	11, 97	0, 100
Week 26		
n	15	12
Mean (SD)	76.7 (21.0)	85.3 (9.8)
Median	83.3	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.6.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 90.0	78.3, 91.7
Min, Max	33, 100	70, 100
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	-1.3 (12.3)	13.7 (34.4)
Median	3.3	0.4
25th, 75th Percentile	-6.7, 6.7	-10.0, 27.8
Min, Max	-30, 13	-20, 80
Week 52		
n	16	12
Mean (SD)	73.4 (21.6)	81.7 (18.6)
Median	80.0	88.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.6.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	58.9, 86.7	75.0, 95.0
Min, Max	20, 100	43, 100
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	-4.6 (10.3)	5.4 (23.4)
Median	-3.3	1.7
25th, 75th Percentile	-10.0, 2.0	-6.7, 7.0
Min, Max	-27, 13	-20, 73
Difference in change from baseline (95%CI)		9.94 (-5.55, 25.43)
P-value ^b		0.1905
Hedges'g (95% CI) ^c		0.56 (-0.21, 1.32)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.6.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Physical Abilities Score		
Baseline		
n	12	16
Mean (SD)	81.1 (14.9)	76.2 (19.1)
Median	85.9	74.2
25th, 75th Percentile	70.8, 91.1	65.0, 93.9
Min, Max	48, 100	33, 100
Week 26		
n	13	19
Mean (SD)	77.8 (23.4)	77.8 (20.3)
Median	83.3	85.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.6.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 90.5	60.0, 93.3
Min, Max	13, 100	40, 100
Change from baseline to Week 26 ^a		
n	11	16
Mean (SD)	1.8 (19.9)	-1.8 (21.6)
Median	0.0	-1.7
25th, 75th Percentile	-18.3, 20.8	-10.6, 4.9
Min, Max	-28, 38	-43, 60
Week 52		
n	14	18
Mean (SD)	85.6 (14.3)	76.5 (15.8)
Median	88.3	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.2.6.101
Infant Toddler Quality of Life (ITQoL): Physical Abilities Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	77.8, 96.3	70.0, 86.7
Min, Max	50, 100	41, 100
Change from baseline to Week 52 ^a		
n	12	15
Mean (SD)	2.4 (22.3)	-0.3 (23.7)
Median	1.7	6.7
25th, 75th Percentile	-8.5, 9.7	-13.3, 10.2
Min, Max	-44, 49	-54, 40
Difference in change from baseline (95%CI)		-2.66 (-21.08, 15.75)
P-value ^b		0.7683
Hedges'g (95% CI) ^c		-0.11 (-0.87, 0.65)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.2582

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.2.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Growth and Development Score		
Baseline		
n	13	17
Mean (SD)	81.2 (14.2)	72.1 (22.2)
Median	85.0	80.0
25th, 75th Percentile	70.0, 90.0	60.0, 87.5
Min, Max	55, 100	20, 100
Week 26		
n	12	16
Mean (SD)	83.8 (9.8)	72.8 (19.0)
Median	83.8	78.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.2.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	77.5, 90.0	57.5, 90.0
Min, Max	63, 98	38, 95
Change from baseline to Week 26 ^a		
n	12	16
Mean (SD)	1.7 (9.0)	1.6 (20.8)
Median	-1.3	-2.5
25th, 75th Percentile	-3.8, 10.0	-11.3, 6.3
Min, Max	-13, 15	-33, 53
Week 52		
n	13	15
Mean (SD)	84.0 (11.6)	79.6 (21.5)
Median	85.0	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.003.002.101_qs_sum_ovr_qol_grow_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 92.5	70.0, 95.0
Min, Max	55, 100	20, 98
Change from baseline to Week 52 ^a		
n	13	15
Mean (SD)	2.9 (10.2)	6.1 (22.3)
Median	0.0	2.5
25th, 75th Percentile	-5.0, 7.5	-5.0, 10.0
Min, Max	-15, 23	-33, 73
Difference in change from baseline (95%CI)		3.20 (-10.16, 16.56)
P-value ^b		0.6231
Hedges'g (95% CI) ^c		0.17 (-0.57, 0.92)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.003.002.101_qs_sum_ovr_qol_grow_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Growth and Development Score		
Baseline		
n	18	15
Mean (SD)	79.5 (12.1)	83.7 (14.0)
Median	81.3	82.5
25th, 75th Percentile	72.5, 87.5	77.5, 97.5
Min, Max	50, 98	58, 100
Week 26		
n	16	15
Mean (SD)	84.2 (15.2)	86.8 (11.3)
Median	87.5	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	81.3, 92.5	77.5, 97.5
Min, Max	48, 100	65, 100
Change from baseline to Week 26 ^a		
n	15	15
Mean (SD)	2.2 (12.5)	3.2 (11.3)
Median	5.0	2.5
25th, 75th Percentile	0.0, 10.0	-5.0, 7.5
Min, Max	-30, 20	-23, 23
Week 52		
n	17	15
Mean (SD)	85.4 (8.4)	81.8 (18.4)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 90.0	62.5, 100.0
Min, Max	70, 100	43, 100
Change from baseline to Week 52 ^a		
n	16	15
Mean (SD)	3.3 (9.6)	-1.8 (11.7)
Median	5.0	0.0
25th, 75th Percentile	-2.0, 10.0	-10.0, 5.0
Min, Max	-15, 20	-25, 18
Difference in change from baseline (95%CI)		-5.18 (-13.02, 2.66)
P-value ^b		0.1869
Hedges'g (95% CI) ^c		-0.47 (-1.18, 0.25)
P-value for interaction term, treatment * [Sex]		0.2718

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Growth and Development Score		
Baseline		
n	7	7
Mean (SD)	75.4 (15.1)	73.9 (20.5)
Median	77.5	72.5
25th, 75th Percentile	62.5, 85.0	60.0, 92.5
Min, Max	55, 100	43, 100
Week 26		
n	7	7
Mean (SD)	81.1 (11.0)	77.9 (16.0)
Median	80.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	77.5, 90.0	57.5, 90.0
Min, Max	63, 98	58, 95
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	5.7 (8.9)	3.9 (23.0)
Median	7.5	-2.5
25th, 75th Percentile	-2.5, 15.0	-15.0, 7.5
Min, Max	-5, 15	-15, 53
Week 52		
n	7	6
Mean (SD)	83.2 (8.5)	81.9 (16.5)
Median	80.0	88.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.2.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 85.0	72.5, 93.8
Min, Max	73, 100	53, 95
Change from baseline to Week 52 ^a		
n	7	6
Mean (SD)	7.9 (10.1)	2.7 (11.1)
Median	7.5	-1.3
25th, 75th Percentile	0.0, 17.5	-5.0, 10.0
Min, Max	-5, 23	-8, 21
Difference in change from baseline (95%CI)		-5.15 (-18.14, 7.84)
P-value ^b		0.4016
Hedges'g (95% CI) ^c		-0.45 (-1.55, 0.66)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.2.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Growth and Development Score		
Baseline		
n	8	8
Mean (SD)	76.9 (13.9)	79.7 (14.7)
Median	81.3	82.5
25th, 75th Percentile	68.8, 86.3	68.8, 88.8
Min, Max	50, 93	58, 100
Week 26		
n	7	8
Mean (SD)	78.9 (21.9)	81.6 (11.3)
Median	85.0	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.2.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	50.0, 100.0	76.3, 90.0
Min, Max	48, 100	65, 100
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	-1.7 (14.5)	1.9 (7.9)
Median	1.3	0.0
25th, 75th Percentile	0.0, 7.5	-5.0, 6.3
Min, Max	-30, 10	-5, 18
Week 52		
n	8	8
Mean (SD)	82.5 (9.7)	84.1 (16.7)
Median	83.8	90.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.2.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 86.3	70.0, 97.5
Min, Max	70, 100	58, 100
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	1.8 (13.0)	4.4 (7.9)
Median	0.0	2.5
25th, 75th Percentile	-12.5, 15.0	0.0, 10.0
Min, Max	-15, 20	-8, 18
Difference in change from baseline (95%CI)		2.59 (-9.19, 14.37)
P-value ^b		0.6428
Hedges'g (95% CI) ^c		0.23 (-0.79, 1.25)
P-value for interaction term, treatment *[Sex]		0.3449

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Growth and Development Score		
Baseline		
n	24	21
Mean (SD)	81.0 (12.8)	80.7 (17.4)
Median	85.0	82.5
25th, 75th Percentile	71.3, 87.5	77.5, 92.5
Min, Max	50, 100	43, 100
Week 26		
n	22	20
Mean (SD)	87.5 (10.6)	82.5 (17.4)
Median	87.5	86.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 95.0	76.3, 96.3
Min, Max	50, 100	48, 100
Change from baseline to Week 26 ^a		
n	21	20
Mean (SD)	4.6 (8.3)	2.0 (17.2)
Median	2.5	0.0
25th, 75th Percentile	0.0, 12.5	-5.0, 6.3
Min, Max	-10, 20	-33, 53
Week 52		
n	23	19
Mean (SD)	86.7 (8.4)	84.7 (15.9)
Median	87.5	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 92.5	77.5, 97.5
Min, Max	70, 100	53, 100
Change from baseline to Week 52 ^a		
n	22	19
Mean (SD)	3.8 (9.6)	1.9 (10.7)
Median	3.8	2.5
25th, 75th Percentile	0.0, 10.0	-7.5, 7.5
Min, Max	-15, 23	-18, 21
Difference in change from baseline (95%CI)		-1.84 (-8.25, 4.57)
P-value ^b		0.5643
Hedges'g (95% CI) ^c		-0.18 (-0.79, 0.44)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.3.3.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Growth and Development Score		
Baseline		
n	7	11
Mean (SD)	77.3 (13.6)	71.4 (22.4)
Median	77.5	77.5
25th, 75th Percentile	70.0, 90.0	60.0, 87.5
Min, Max	55, 96	20, 100
Week 26		
n	6	11
Mean (SD)	71.3 (13.6)	74.3 (15.9)
Median	77.5	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	62.5, 80.0	65.0, 90.0
Min, Max	48, 83	38, 90
Change from baseline to Week 26 ^a		
n	6	11
Mean (SD)	-7.3 (14.5)	3.0 (16.2)
Median	-7.5	-2.5
25th, 75th Percentile	-13.9, 7.5	-5.0, 7.5
Min, Max	-30, 8	-15, 40
Week 52		
n	7	11
Mean (SD)	78.6 (12.0)	73.9 (24.2)
Median	82.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.3.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.5, 85.0	62.5, 92.5
Min, Max	55, 93	20, 95
Change from baseline to Week 52 ^a		
n	7	11
Mean (SD)	1.2 (10.7)	2.5 (26.9)
Median	0.0	2.5
25th, 75th Percentile	-5.0, 10.0	-7.5, 10.0
Min, Max	-15, 18	-33, 73
Difference in change from baseline (95%CI)		1.28 (-18.16, 20.71)
P-value ^b		0.8901
Hedges'g (95% CI) ^c		0.05 (-0.89, 1.00)
P-value for interaction term, treatment * [Ethnicity]		0.7146

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.3.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Growth and Development Score		
Baseline		
n	12	8
Mean (SD)	77.5 (14.3)	73.4 (19.3)
Median	81.3	75.0
25th, 75th Percentile	67.5, 86.3	58.8, 88.8
Min, Max	50, 100	43, 100
Week 26		
n	11	8
Mean (SD)	84.8 (14.1)	80.3 (16.6)
Median	85.0	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 97.5	66.3, 95.0
Min, Max	50, 100	58, 100
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	5.5 (7.4)	6.9 (20.7)
Median	5.0	0.0
25th, 75th Percentile	0.0, 12.5	-3.8, 12.5
Min, Max	-5, 15	-15, 53
Week 52		
n	12	7
Mean (SD)	84.0 (9.4)	82.3 (20.2)
Median	85.0	93.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 86.3	57.5, 100.0
Min, Max	70, 100	53, 100
Change from baseline to Week 52 ^a		
n	11	7
Mean (SD)	4.1 (12.5)	4.5 (11.5)
Median	0.0	0.0
25th, 75th Percentile	-5.0, 15.0	-7.5, 17.5
Min, Max	-15, 23	-8, 21
Difference in change from baseline (95%CI)		0.37 (-12.06, 12.80)
P-value ^b		0.9500
Hedges'g (95% CI) ^c		0.03 (-0.92, 0.98)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.3.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Growth and Development Score		
Baseline		
n	3	7
Mean (SD)	70.8 (13.8)	81.1 (14.9)
Median	77.5	82.5
25th, 75th Percentile	55.0, 80.0	62.5, 92.5
Min, Max	55, 80	60, 100
Week 26		
n	3	7
Mean (SD)	62.5 (15.0)	79.3 (9.7)
Median	62.5	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.3.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	47.5, 77.5	70.0, 90.0
Min, Max	48, 78	65, 90
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	-8.3 (19.4)	-1.8 (7.6)
Median	-2.5	-2.5
25th, 75th Percentile	-30.0, 7.5	-5.0, 5.0
Min, Max	-30, 8	-15, 8
Week 52		
n	3	7
Mean (SD)	78.3 (5.2)	83.9 (12.1)
Median	80.0	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.3.3.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.5, 82.5	72.5, 95.0
Min, Max	73, 83	63, 95
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	7.5 (9.0)	2.9 (6.7)
Median	5.0	2.5
25th, 75th Percentile	0.0, 17.5	-5.0, 10.0
Min, Max	0, 18	-5, 13
Difference in change from baseline (95%CI)		-4.64 (-16.31, 7.03)
P-value ^b		0.3858
Hedges'g (95% CI) ^c		-0.57 (-1.94, 0.83)
P-value for interaction term, treatment * [Ethnicity]		0.5855

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Growth and Development Score		
Baseline		
n	3	8
Mean (SD)	65.0 (11.5)	80.9 (13.9)
Median	62.5	83.8
25th, 75th Percentile	55.0, 77.5	70.0, 90.0
Min, Max	55, 78	60, 100
Week 26		
n	4	8
Mean (SD)	82.5 (16.2)	80.0 (9.2)
Median	83.8	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 95.0	73.8, 87.5
Min, Max	63, 100	65, 90
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	11.7 (3.8)	-0.9 (6.9)
Median	12.5	0.0
25th, 75th Percentile	7.5, 15.0	-3.8, 3.8
Min, Max	8, 15	-15, 8
Week 52		
n	4	8
Mean (SD)	85.6 (11.3)	84.4 (12.2)
Median	85.0	88.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	78.8, 92.5	75.0, 95.0
Min, Max	73, 100	63, 95
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	15.8 (7.6)	3.4 (9.2)
Median	17.5	2.5
25th, 75th Percentile	7.5, 22.5	-5.0, 11.3
Min, Max	8, 23	-8, 18
Difference in change from baseline (95%CI)		-12.40 (-25.93, 1.14)
P-value ^b		0.0682
Hedges'g (95% CI) ^c		-1.28 (-2.70, 0.20)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.3.4.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Growth and Development Score		
Baseline		
n	12	7
Mean (SD)	79.0 (13.5)	72.5 (20.6)
Median	82.5	72.5
25th, 75th Percentile	68.8, 86.3	57.5, 92.5
Min, Max	50, 100	43, 100
Week 26		
n	10	7
Mean (SD)	79.0 (17.6)	79.6 (17.8)
Median	81.3	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	77.5, 90.0	57.5, 95.0
Min, Max	48, 100	58, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-0.5 (12.1)	7.1 (22.5)
Median	0.0	-2.5
25th, 75th Percentile	-2.5, 7.5	-5.0, 17.5
Min, Max	-30, 15	-15, 53
Week 52		
n	11	6
Mean (SD)	81.8 (8.2)	81.5 (21.3)
Median	80.0	89.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 85.0	57.5, 100.0
Min, Max	70, 100	53, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	1.8 (10.8)	4.0 (9.8)
Median	0.0	1.3
25th, 75th Percentile	-5.0, 12.5	0.0, 7.5
Min, Max	-15, 20	-8, 21
Difference in change from baseline (95%CI)		2.14 (-9.21, 13.49)
P-value ^b		0.6935
Hedges'g (95% CI) ^c		0.19 (-0.81, 1.19)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.0957

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Growth and Development Score		
Baseline		
n	3	8
Mean (SD)	65.0 (11.5)	80.9 (13.9)
Median	62.5	83.8
25th, 75th Percentile	55.0, 77.5	70.0, 90.0
Min, Max	55, 78	60, 100
Week 26		
n	4	8
Mean (SD)	82.5 (16.2)	80.0 (9.2)
Median	83.8	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.0, 95.0	73.8, 87.5
Min, Max	63, 100	65, 90
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	11.7 (3.8)	-0.9 (6.9)
Median	12.5	0.0
25th, 75th Percentile	7.5, 15.0	-3.8, 3.8
Min, Max	8, 15	-15, 8
Week 52		
n	4	8
Mean (SD)	85.6 (11.3)	84.4 (12.2)
Median	85.0	88.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.003.004.102_qs_sum_ovr_qol_grow_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.4.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	78.8, 92.5	75.0, 95.0
Min, Max	73, 100	63, 95
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	15.8 (7.6)	3.4 (9.2)
Median	17.5	2.5
25th, 75th Percentile	7.5, 22.5	-5.0, 11.3
Min, Max	8, 23	-8, 18
Difference in change from baseline (95%CI)		-12.40 (-25.93, 1.14)
P-value ^b		0.0682
Hedges'g (95% CI) ^c		-1.28 (-2.70, 0.20)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Growth and Development Score		
Baseline		
n	12	7
Mean (SD)	79.0 (13.5)	72.5 (20.6)
Median	82.5	72.5
25th, 75th Percentile	68.8, 86.3	57.5, 92.5
Min, Max	50, 100	43, 100
Week 26		
n	10	7
Mean (SD)	79.0 (17.6)	79.6 (17.8)
Median	81.3	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.4.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	77.5, 90.0	57.5, 95.0
Min, Max	48, 100	58, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-0.5 (12.1)	7.1 (22.5)
Median	0.0	-2.5
25th, 75th Percentile	-2.5, 7.5	-5.0, 17.5
Min, Max	-30, 15	-15, 53
Week 52		
n	11	6
Mean (SD)	81.8 (8.2)	81.5 (21.3)
Median	80.0	89.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.4.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 85.0	57.5, 100.0
Min, Max	70, 100	53, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	1.8 (10.8)	4.0 (9.8)
Median	0.0	1.3
25th, 75th Percentile	-5.0, 12.5	0.0, 7.5
Min, Max	-15, 20	-8, 21
Difference in change from baseline (95%CI)		2.14 (-9.21, 13.49)
P-value ^b		0.6935
Hedges'g (95% CI) ^c		0.19 (-0.81, 1.19)
P-value for interaction term, treatment *[Cohort 1 Age Stratum]		0.0957

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Growth and Development Score		
Baseline		
n	11	7
Mean (SD)	80.9 (14.3)	75.7 (23.8)
Median	85.0	82.5
25th, 75th Percentile	70.0, 92.5	52.5, 100.0
Min, Max	50, 100	43, 100
Week 26		
n	11	7
Mean (SD)	85.5 (14.0)	76.8 (21.7)
Median	87.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 97.5	57.5, 95.0
Min, Max	50, 100	38, 95
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	3.5 (7.4)	1.1 (23.3)
Median	1.3	-5.0
25th, 75th Percentile	-2.5, 10.0	-15.0, -2.5
Min, Max	-8, 15	-15, 53
Week 52		
n	12	6
Mean (SD)	84.8 (9.9)	73.3 (31.0)
Median	85.0	86.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 91.3	52.5, 95.0
Min, Max	70, 100	20, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	2.5 (10.7)	-7.9 (12.6)
Median	0.0	-5.0
25th, 75th Percentile	0.0, 12.5	-7.5, 0.0
Min, Max	-15, 20	-33, 3
Difference in change from baseline (95%CI)		-10.42 (-22.73, 1.89)
P-value ^b		0.0914
Hedges'g (95% CI) ^c		-0.87 (-1.90, 0.19)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Growth and Development Score		
Baseline		
n	20	25
Mean (SD)	79.8 (12.3)	78.0 (18.6)
Median	81.3	82.5
25th, 75th Percentile	71.3, 87.5	72.5, 92.5
Min, Max	55, 100	20, 100
Week 26		
n	17	24
Mean (SD)	83.1 (12.5)	80.4 (16.0)
Median	87.5	83.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 90.0	72.5, 92.5
Min, Max	48, 98	48, 100
Change from baseline to Week 26 ^a		
n	17	24
Mean (SD)	1.1 (12.7)	2.7 (14.7)
Median	2.5	2.5
25th, 75th Percentile	-5.0, 7.5	-3.8, 7.5
Min, Max	-30, 20	-33, 40
Week 52		
n	18	24
Mean (SD)	84.9 (9.9)	82.6 (16.2)
Median	86.3	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 92.5	71.3, 95.0
Min, Max	55, 98	43, 100
Change from baseline to Week 52 ^a		
n	18	24
Mean (SD)	3.5 (9.3)	4.6 (18.4)
Median	5.0	2.5
25th, 75th Percentile	-5.0, 10.0	-3.8, 10.0
Min, Max	-15, 23	-25, 73
Difference in change from baseline (95%CI)		1.10 (-7.72, 9.93)
P-value ^b		0.8012
Hedges'g (95% CI) ^c		0.07 (-0.54, 0.68)
P-value for interaction term, treatment * [Baseline AGV Category]		0.1809

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
<= 4.5		
ITQoL : Growth and Development Score		
Baseline		
n	10	6
Mean (SD)	79.5 (14.2)	79.6 (23.5)
Median	82.5	87.5
25th, 75th Percentile	70.0, 87.5	60.0, 100.0
Min, Max	50, 100	43, 100
Week 26		
n	10	6
Mean (SD)	85.3 (14.8)	83.3 (14.3)
Median	87.5	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 97.5	77.5, 95.0
Min, Max	50, 100	58, 95
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	4.7 (6.7)	3.8 (24.3)
Median	2.5	-3.8
25th, 75th Percentile	0.0, 10.0	-5.0, -2.5
Min, Max	-3, 15	-15, 53
Week 52		
n	11	5
Mean (SD)	83.9 (9.8)	84.0 (18.6)
Median	85.0	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.3.5.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.0, 87.5	85.0, 95.0
Min, Max	70, 100	53, 100
Change from baseline to Week 52 ^a		
n	10	5
Mean (SD)	2.8 (11.3)	-3.0 (4.1)
Median	0.0	-5.0
25th, 75th Percentile	0.0, 12.5	-5.0, 0.0
Min, Max	-15, 20	-8, 3
Difference in change from baseline (95%CI)		-5.75 (-17.17, 5.67)
P-value ^b		0.2964
Hedges'g (95% CI) ^c		-0.56 (-1.65, 0.54)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Growth and Development Score		
Baseline		
n	5	9
Mean (SD)	69.5 (11.9)	75.3 (12.8)
Median	67.5	77.5
25th, 75th Percentile	62.5, 77.5	62.5, 85.0
Min, Max	55, 85	58, 93
Week 26		
n	4	9
Mean (SD)	66.9 (15.1)	77.5 (12.9)
Median	70.0	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.5.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	55.0, 78.8	70.0, 85.0
Min, Max	48, 80	58, 100
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	-3.1 (19.7)	2.2 (9.1)
Median	1.3	2.5
25th, 75th Percentile	-17.5, 11.3	0.0, 7.5
Min, Max	-30, 15	-15, 18
Week 52		
n	4	9
Mean (SD)	80.0 (5.4)	82.6 (15.6)
Median	81.3	90.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.003.005.102_qs_sum_ovr_qol_grow_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.5.102
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	76.3, 83.8	72.5, 95.0
Min, Max	73, 85	58, 100
Change from baseline to Week 52 ^a		
n	4	9
Mean (SD)	10.0 (12.4)	7.4 (9.0)
Median	11.3	7.5
25th, 75th Percentile	0.0, 20.0	2.5, 12.5
Min, Max	-5, 23	-8, 21
Difference in change from baseline (95%CI)		-2.64 (-15.95, 10.67)
P-value ^b		0.6710
Hedges'g (95% CI) ^c		-0.24 (-1.42, 0.94)
P-value for interaction term, treatment * [Baseline AGV Category]		0.7009

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.6.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Growth and Development Score		
Baseline		
n	18	13
Mean (SD)	77.5 (14.2)	73.3 (23.7)
Median	78.8	82.5
25th, 75th Percentile	67.5, 87.5	60.0, 92.5
Min, Max	50, 100	20, 100
Week 26		
n	15	12
Mean (SD)	80.2 (16.0)	77.5 (15.5)
Median	82.5	77.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.3.6.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	77.5, 90.0	62.5, 92.5
Min, Max	48, 100	58, 100
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	1.7 (12.4)	5.2 (20.7)
Median	2.5	-2.5
25th, 75th Percentile	-2.5, 10.0	-5.0, 7.5
Min, Max	-30, 20	-15, 53
Week 52		
n	16	12
Mean (SD)	83.8 (8.5)	85.9 (15.4)
Median	85.0	93.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.6.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 88.8	78.8, 95.0
Min, Max	70, 100	53, 100
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	4.8 (11.0)	10.1 (21.3)
Median	2.5	5.0
25th, 75th Percentile	0.0, 13.8	-2.5, 11.3
Min, Max	-15, 23	-8, 73
Difference in change from baseline (95%CI)		5.26 (-9.10, 19.62)
P-value ^b		0.4478
Hedges'g (95% CI) ^c		0.31 (-0.44, 1.07)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.6.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Growth and Development Score		
Baseline		
n	13	19
Mean (SD)	84.0 (10.0)	80.4 (15.9)
Median	85.0	82.5
25th, 75th Percentile	75.0, 87.5	77.5, 92.5
Min, Max	70, 100	43, 100
Week 26		
n	13	19
Mean (SD)	88.5 (6.2)	80.9 (18.3)
Median	87.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.3.6.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 90.0	75.0, 95.0
Min, Max	80, 100	38, 100
Change from baseline to Week 26 ^a		
n	12	19
Mean (SD)	2.4 (9.2)	0.5 (13.7)
Median	2.5	0.0
25th, 75th Percentile	-3.8, 10.0	-5.0, 5.0
Min, Max	-14, 15	-33, 23
Week 52		
n	14	18
Mean (SD)	86.1 (11.3)	77.2 (21.8)
Median	88.8	83.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.3.6.101
Infant Toddler Quality of Life (ITQoL): Growth and Development Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.0, 92.5	60.0, 95.0
Min, Max	55, 100	20, 100
Change from baseline to Week 52 ^a		
n	13	18
Mean (SD)	1.0 (7.7)	-3.2 (13.4)
Median	2.5	1.3
25th, 75th Percentile	-5.0, 7.5	-10.0, 5.0
Min, Max	-15, 10	-33, 18
Difference in change from baseline (95%CI)		-4.24 (-12.70, 4.22)
P-value ^b		0.3138
Hedges'g (95% CI) ^c		-0.36 (-1.08, 0.36)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.1998

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.2.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Bodily Pain Score		
Baseline		
n	13	17
Mean (SD)	85.3 (12.3)	84.3 (11.0)
Median	91.7	83.3
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	67, 100	75, 100
Week 26		
n	12	16
Mean (SD)	84.0 (12.5)	83.3 (19.7)
Median	83.3	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.2.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 95.8	70.8, 100.0
Min, Max	67, 100	42, 100
Change from baseline to Week 26 ^a		
n	12	16
Mean (SD)	-0.7 (12.5)	-1.0 (19.0)
Median	0.0	0.0
25th, 75th Percentile	-8.3, 8.3	-12.5, 12.5
Min, Max	-25, 17	-33, 25
Week 52		
n	13	15
Mean (SD)	81.4 (16.0)	81.1 (21.2)
Median	75.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.2.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 100.0	75.0, 100.0
Min, Max	50, 100	17, 100
Change from baseline to Week 52 ^a		
n	13	15
Mean (SD)	-3.8 (15.1)	-3.9 (26.1)
Median	-8.3	0.0
25th, 75th Percentile	-16.7, 8.3	-8.3, 8.3
Min, Max	-25, 25	-83, 25
Difference in change from baseline (95%CI)		-0.04 (-16.98, 16.89)
P-value ^b		0.9960
Hedges'g (95% CI) ^c		0.00 (-0.74, 0.74)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.2.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Bodily Pain Score		
Baseline		
n	18	15
Mean (SD)	75.2 (16.7)	92.2 (13.2)
Median	75.0	100.0
25th, 75th Percentile	66.7, 83.3	83.3, 100.0
Min, Max	33, 100	58, 100
Week 26		
n	16	15
Mean (SD)	81.3 (18.6)	87.8 (12.5)
Median	87.5	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 100.0	75.0, 100.0
Min, Max	42, 100	67, 100
Change from baseline to Week 26 ^a		
n	15	15
Mean (SD)	5.8 (24.0)	-4.4 (16.3)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 25.0	-16.7, 8.3
Min, Max	-21, 50	-33, 25
Week 52		
n	17	15
Mean (SD)	76.0 (16.9)	90.6 (12.9)
Median	75.0	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 83.3	83.3, 100.0
Min, Max	42, 100	58, 100
Change from baseline to Week 52 ^a		
n	16	15
Mean (SD)	-0.8 (26.1)	-1.7 (17.6)
Median	0.0	0.0
25th, 75th Percentile	-25.0, 14.6	-8.3, 0.0
Min, Max	-33, 50	-42, 42
Difference in change from baseline (95%CI)		-0.89 (-17.35, 15.58)
P-value ^b		0.9131
Hedges'g (95% CI) ^c		-0.04 (-0.74, 0.67)
P-value for interaction term, treatment * [Sex]		0.9419

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Bodily Pain Score		
Baseline		
n	7	7
Mean (SD)	83.3 (11.8)	85.7 (13.4)
Median	83.3	75.0
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	67, 100	75, 100
Week 26		
n	7	7
Mean (SD)	86.9 (14.3)	96.4 (9.4)
Median	91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 100.0	100.0, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	3.6 (10.6)	10.7 (13.4)
Median	8.3	0.0
25th, 75th Percentile	0.0, 8.3	0.0, 25.0
Min, Max	-17, 17	0, 25
Week 52		
n	7	6
Mean (SD)	84.5 (13.1)	81.9 (11.1)
Median	83.3	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.002.102_qs_sum_ovr_qol_pain_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 100.0	75.0, 83.3
Min, Max	67, 100	67, 100
Change from baseline to Week 52 ^a		
n	7	6
Mean (SD)	1.2 (16.3)	-5.6 (16.4)
Median	8.3	0.0
25th, 75th Percentile	-8.3, 8.3	-16.7, 8.3
Min, Max	-25, 25	-33, 8
Difference in change from baseline (95%CI)		-6.75 (-26.73, 13.24)
P-value ^b		0.4730
Hedges'g (95% CI) ^c		-0.38 (-1.48, 0.73)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.002.102_qs_sum_ovr_qol_pain_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Bodily Pain Score		
Baseline		
n	8	8
Mean (SD)	81.2 (14.6)	89.6 (15.9)
Median	83.3	100.0
25th, 75th Percentile	79.2, 87.5	79.2, 100.0
Min, Max	50, 100	58, 100
Week 26		
n	7	8
Mean (SD)	90.5 (12.2)	90.6 (11.3)
Median	91.7	95.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.002.102_qs_sum_ovr_qol_pain_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	83.3, 100.0	79.2, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	9.7 (22.6)	1.0 (13.7)
Median	4.2	0.0
25th, 75th Percentile	0.0, 16.7	-8.3, 8.3
Min, Max	-17, 50	-17, 25
Week 52		
n	8	8
Mean (SD)	80.2 (19.4)	91.7 (14.8)
Median	83.3	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.002.102_qs_sum_ovr_qol_pain_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.2.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 95.8	87.5, 100.0
Min, Max	42, 100	58, 100
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	-3.6 (24.9)	2.1 (23.0)
Median	0.0	0.0
25th, 75th Percentile	-25.0, 8.3	0.0, 8.3
Min, Max	-33, 42	-42, 42
Difference in change from baseline (95%CI)		5.65 (-21.11, 32.41)
P-value ^b		0.6556
Hedges'g (95% CI) ^c		0.22 (-0.80, 1.24)
P-value for interaction term, treatment * [Sex]		0.4402

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Bodily Pain Score		
Baseline		
n	24	21
Mean (SD)	78.1 (14.3)	86.1 (11.0)
Median	75.0	83.3
25th, 75th Percentile	75.0, 87.5	75.0, 100.0
Min, Max	33, 100	75, 100
Week 26		
n	22	20
Mean (SD)	83.3 (14.3)	85.0 (18.7)
Median	83.3	95.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.003.101_qs_sum_ovr_qol_pain_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 100.0	70.8, 100.0
Min, Max	58, 100	42, 100
Change from baseline to Week 26 ^a		
n	21	20
Mean (SD)	4.8 (17.2)	-1.2 (19.2)
Median	0.0	0.0
25th, 75th Percentile	0.0, 8.3	-12.5, 12.5
Min, Max	-17, 42	-33, 25
Week 52		
n	23	19
Mean (SD)	77.9 (17.9)	89.0 (10.8)
Median	83.3	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.3.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 91.7	83.3, 100.0
Min, Max	42, 100	67, 100
Change from baseline to Week 52 ^a		
n	22	19
Mean (SD)	-1.5 (20.4)	2.2 (11.1)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 8.3	-8.3, 8.3
Min, Max	-33, 50	-17, 25
Difference in change from baseline (95%CI)		3.71 (-6.51, 13.93)
P-value ^b		0.4658
Hedges'g (95% CI) ^c		0.22 (-0.40, 0.83)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.3.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Bodily Pain Score		
Baseline		
n	7	11
Mean (SD)	83.9 (20.2)	91.7 (14.9)
Median	91.7	100.0
25th, 75th Percentile	62.5, 100.0	75.0, 100.0
Min, Max	50, 100	58, 100
Week 26		
n	6	11
Mean (SD)	79.2 (22.8)	86.4 (12.5)
Median	83.3	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.3.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 100.0	75.0, 100.0
Min, Max	42, 100	67, 100
Change from baseline to Week 26 ^a		
n	6	11
Mean (SD)	-3.5 (27.7)	-5.3 (14.6)
Median	-12.5	0.0
25th, 75th Percentile	-20.8, 0.0	-16.7, 0.0
Min, Max	-25, 50	-33, 17
Week 52		
n	7	11
Mean (SD)	79.8 (11.6)	80.3 (25.9)
Median	75.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.3.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 91.7	66.7, 100.0
Min, Max	67, 100	17, 100
Change from baseline to Week 52 ^a		
n	7	11
Mean (SD)	-4.2 (26.7)	-11.4 (32.3)
Median	-8.3	0.0
25th, 75th Percentile	-25.0, 12.5	-33.3, 0.0
Min, Max	-33, 42	-83, 42
Difference in change from baseline (95%CI)		-7.20 (-38.29, 23.90)
P-value ^b		0.6303
Hedges'g (95% CI) ^c		-0.23 (-1.17, 0.73)
P-value for interaction term, treatment * [Ethnicity]		0.3842

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Bodily Pain Score		
Baseline		
n	12	8
Mean (SD)	83.3 (9.4)	85.4 (12.4)
Median	83.3	79.2
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	67, 100	75, 100
Week 26		
n	11	8
Mean (SD)	88.6 (11.9)	96.9 (8.8)
Median	91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 100.0	100.0, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	5.0 (9.8)	11.5 (14.7)
Median	8.3	12.5
25th, 75th Percentile	0.0, 8.3	0.0, 25.0
Min, Max	-17, 17	-8, 25
Week 52		
n	12	7
Mean (SD)	82.6 (17.9)	90.5 (10.1)
Median	83.3	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.4.3.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 100.0	83.3, 100.0
Min, Max	42, 100	75, 100
Change from baseline to Week 52 ^a		
n	11	7
Mean (SD)	-2.3 (17.1)	3.6 (6.6)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 8.3	0.0, 8.3
Min, Max	-33, 25	0, 17
Difference in change from baseline (95%CI)		5.84 (-6.44, 18.13)
P-value ^b		0.3247
Hedges'g (95% CI) ^c		0.39 (-0.57, 1.35)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Bodily Pain Score		
Baseline		
n	3	7
Mean (SD)	77.8 (25.5)	90.5 (17.0)
Median	83.3	100.0
25th, 75th Percentile	50.0, 100.0	75.0, 100.0
Min, Max	50, 100	58, 100
Week 26		
n	3	7
Mean (SD)	88.9 (19.2)	89.3 (11.5)
Median	100.0	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	66.7, 100.0	75.0, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	11.1 (34.7)	-1.2 (10.1)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 50.0	-8.3, 0.0
Min, Max	-17, 50	-17, 17
Week 52		
n	3	7
Mean (SD)	80.6 (9.6)	84.5 (17.0)
Median	75.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.003.102_qs_sum_ovr_qol_pain_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.3.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 91.7	66.7, 100.0
Min, Max	75, 92	58, 100
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	2.8 (34.7)	-6.0 (27.9)
Median	-8.3	0.0
25th, 75th Percentile	-25.0, 41.7	-33.3, 8.3
Min, Max	-25, 42	-42, 42
Difference in change from baseline (95%CI)		-8.73 (-56.11, 38.64)
P-value ^b		0.6820
Hedges'g (95% CI) ^c		-0.26 (-1.62, 1.10)
P-value for interaction term, treatment * [Ethnicity]		0.4114

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Bodily Pain Score		
Baseline		
n	3	8
Mean (SD)	83.3 (8.3)	88.5 (16.6)
Median	83.3	100.0
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	75, 92	58, 100
Week 26		
n	4	8
Mean (SD)	85.4 (17.2)	91.7 (11.8)
Median	87.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.004.101_qs_sum_ovr_qol_pain_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 100.0	79.2, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	-2.8 (12.7)	3.1 (12.6)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 8.3	0.0, 8.3
Min, Max	-17, 8	-17, 25
Week 52		
n	4	8
Mean (SD)	85.4 (17.2)	85.4 (15.9)
Median	87.5	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 100.0	75.0, 100.0
Min, Max	67, 100	58, 100
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	-2.8 (9.6)	-3.1 (27.1)
Median	-8.3	0.0
25th, 75th Percentile	-8.3, 8.3	-25.0, 12.5
Min, Max	-8, 8	-42, 42
Difference in change from baseline (95%CI)		-0.35 (-37.57, 36.87)
P-value ^b		0.9836
Hedges'g (95% CI) ^c		-0.01 (-1.34, 1.31)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.004.101_qs_sum_ovr_qol_pain_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Bodily Pain Score		
Baseline		
n	12	7
Mean (SD)	81.9 (14.1)	86.9 (12.6)
Median	83.3	83.3
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	50, 100	75, 100
Week 26		
n	10	7
Mean (SD)	90.0 (11.7)	95.2 (9.4)
Median	91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.004.101_qs_sum_ovr_qol_pain_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	83.3, 100.0	91.7, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	9.2 (17.3)	8.3 (16.0)
Median	8.3	0.0
25th, 75th Percentile	0.0, 16.7	-8.3, 25.0
Min, Max	-17, 50	-8, 25
Week 52		
n	11	6
Mean (SD)	81.1 (16.7)	90.3 (11.1)
Median	83.3	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.4.4.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 91.7	83.3, 100.0
Min, Max	42, 100	75, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-0.8 (22.8)	1.4 (3.4)
Median	0.0	0.0
25th, 75th Percentile	-25.0, 8.3	0.0, 0.0
Min, Max	-33, 42	0, 8
Difference in change from baseline (95%CI)		2.15 (-13.33, 17.62)
P-value ^b		0.7655
Hedges'g (95% CI) ^c		0.11 (-0.89, 1.10)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.8895

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.4.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Bodily Pain Score		
Baseline		
n	3	8
Mean (SD)	83.3 (8.3)	88.5 (16.6)
Median	83.3	100.0
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	75, 92	58, 100
Week 26		
n	4	8
Mean (SD)	85.4 (17.2)	91.7 (11.8)
Median	87.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.4.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 100.0	79.2, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	-2.8 (12.7)	3.1 (12.6)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 8.3	0.0, 8.3
Min, Max	-17, 8	-17, 25
Week 52		
n	4	8
Mean (SD)	85.4 (17.2)	85.4 (15.9)
Median	87.5	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.4.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 100.0	75.0, 100.0
Min, Max	67, 100	58, 100
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	-2.8 (9.6)	-3.1 (27.1)
Median	-8.3	0.0
25th, 75th Percentile	-8.3, 8.3	-25.0, 12.5
Min, Max	-8, 8	-42, 42
Difference in change from baseline (95%CI)		-0.35 (-37.57, 36.87)
P-value ^b		0.9836
Hedges'g (95% CI) ^c		-0.01 (-1.34, 1.31)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Bodily Pain Score		
Baseline		
n	12	7
Mean (SD)	81.9 (14.1)	86.9 (12.6)
Median	83.3	83.3
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	50, 100	75, 100
Week 26		
n	10	7
Mean (SD)	90.0 (11.7)	95.2 (9.4)
Median	91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.004.102_qs_sum_ovr_qol_pain_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	83.3, 100.0	91.7, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	9.2 (17.3)	8.3 (16.0)
Median	8.3	0.0
25th, 75th Percentile	0.0, 16.7	-8.3, 25.0
Min, Max	-17, 50	-8, 25
Week 52		
n	11	6
Mean (SD)	81.1 (16.7)	90.3 (11.1)
Median	83.3	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.4.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 91.7	83.3, 100.0
Min, Max	42, 100	75, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-0.8 (22.8)	1.4 (3.4)
Median	0.0	0.0
25th, 75th Percentile	-25.0, 8.3	0.0, 0.0
Min, Max	-33, 42	0, 8
Difference in change from baseline (95%CI)		2.15 (-13.33, 17.62)
P-value ^b		0.7655
Hedges'g (95% CI) ^c		0.11 (-0.89, 1.10)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.8895

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.5.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Bodily Pain Score		
Baseline		
n	11	7
Mean (SD)	86.4 (11.3)	92.9 (12.2)
Median	83.3	100.0
25th, 75th Percentile	75.0, 100.0	75.0, 100.0
Min, Max	67, 100	75, 100
Week 26		
n	11	7
Mean (SD)	87.9 (12.0)	95.2 (9.4)
Median	91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.5.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 100.0	91.7, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.8 (10.7)	2.4 (17.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 8.3	-8.3, 25.0
Min, Max	-17, 17	-25, 25
Week 52		
n	12	6
Mean (SD)	81.9 (18.1)	76.4 (32.7)
Median	83.3	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.005.101_qs_sum_ovr_qol_pain_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.5.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 100.0	66.7, 100.0
Min, Max	42, 100	17, 100
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-6.1 (17.5)	-19.4 (34.0)
Median	0.0	0.0
25th, 75th Percentile	-25.0, 8.3	-33.3, 0.0
Min, Max	-33, 25	-83, 0
Difference in change from baseline (95%CI)		-13.38 (-39.67, 12.90)
P-value ^b		0.2949
Hedges'g (95% CI) ^c		-0.52 (-1.53, 0.50)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.5.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Bodily Pain Score		
Baseline		
n	20	25
Mean (SD)	75.6 (16.6)	86.7 (12.5)
Median	75.0	83.3
25th, 75th Percentile	66.7, 87.5	75.0, 100.0
Min, Max	33, 100	58, 100
Week 26		
n	17	24
Mean (SD)	78.9 (17.7)	82.6 (17.2)
Median	75.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.5.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 91.7	70.8, 100.0
Min, Max	42, 100	42, 100
Change from baseline to Week 26 ^a		
n	17	24
Mean (SD)	4.2 (23.7)	-4.2 (17.5)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 16.7	-16.7, 8.3
Min, Max	-25, 50	-33, 25
Week 52		
n	18	24
Mean (SD)	75.9 (15.4)	88.2 (12.0)
Median	75.0	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.5.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 83.3	83.3, 100.0
Min, Max	50, 100	58, 100
Change from baseline to Week 52 ^a		
n	18	24
Mean (SD)	0.2 (23.8)	1.4 (16.2)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 12.5	-8.3, 8.3
Min, Max	-33, 50	-42, 42
Difference in change from baseline (95%CI)		1.16 (-11.34, 13.66)
P-value ^b		0.8525
Hedges'g (95% CI) ^c		0.06 (-0.55, 0.67)
P-value for interaction term, treatment * [Baseline AGV Category]		0.2533

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.5.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
ITQoL : Bodily Pain Score		
Baseline		
n	10	6
Mean (SD)	85.0 (11.0)	91.7 (12.9)
Median	83.3	100.0
25th, 75th Percentile	75.0, 91.7	75.0, 100.0
Min, Max	67, 100	75, 100
Week 26		
n	10	6
Mean (SD)	88.3 (12.5)	98.6 (3.4)
Median	91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.5.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 100.0	100.0, 100.0
Min, Max	67, 100	92, 100
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	2.8 (9.3)	6.9 (14.4)
Median	0.0	0.0
25th, 75th Percentile	0.0, 8.3	0.0, 25.0
Min, Max	-17, 17	-8, 25
Week 52		
n	11	5
Mean (SD)	80.3 (18.0)	88.3 (16.2)
Median	83.3	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.5.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	66.7, 100.0	75.0, 100.0
Min, Max	42, 100	67, 100
Change from baseline to Week 52 ^a		
n	10	5
Mean (SD)	-6.7 (18.3)	-6.7 (14.9)
Median	-4.2	0.0
25th, 75th Percentile	-25.0, 8.3	0.0, 0.0
Min, Max	-33, 25	-33, 0
Difference in change from baseline (95%CI)		0.00 (-20.54, 20.54)
P-value ^b		1.0000
Hedges'g (95% CI) ^c		0.00 (-1.07, 1.07)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.5.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Bodily Pain Score		
Baseline		
n	5	9
Mean (SD)	76.7 (16.0)	85.2 (15.5)
Median	83.3	83.3
25th, 75th Percentile	75.0, 83.3	75.0, 100.0
Min, Max	50, 92	58, 100
Week 26		
n	4	9
Mean (SD)	89.6 (15.8)	89.8 (12.3)
Median	95.8	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.5.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	79.2, 100.0	75.0, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	14.6 (27.5)	4.6 (14.5)
Median	12.5	0.0
25th, 75th Percentile	-4.2, 33.3	0.0, 16.7
Min, Max	-17, 50	-17, 25
Week 52		
n	4	9
Mean (SD)	87.5 (10.8)	87.0 (13.3)
Median	87.5	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.4.5.102
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	79.2, 95.8	83.3, 100.0
Min, Max	75, 100	58, 100
Change from baseline to Week 52 ^a		
n	4	9
Mean (SD)	12.5 (21.0)	1.9 (22.7)
Median	8.3	0.0
25th, 75th Percentile	0.0, 25.0	0.0, 8.3
Min, Max	-8, 42	-42, 42
Difference in change from baseline (95%CI)		-10.65 (-40.11, 18.81)
P-value ^b		0.4430
Hedges'g (95% CI) ^c		-0.44 (-1.63, 0.76)
P-value for interaction term, treatment * [Baseline AGV Category]		0.5138

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.6.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category		Placebo (N=32)	Vosoritide (N=32)
Score			
Visit			
Result			
<hr/>			
<= -4			
ITQoL : Bodily Pain Score			
Baseline			
n		18	13
Mean (SD)		81.9 (15.2)	87.8 (14.7)
Median		83.3	100.0
25th, 75th Percentile		66.7, 91.7	75.0, 100.0
Min, Max		50, 100	58, 100
Week 26			
n		15	12
Mean (SD)		86.7 (13.7)	93.8 (10.1)
Median		91.7	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.6.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 100.0	87.5, 100.0
Min, Max	67, 100	75, 100
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	4.4 (20.6)	5.6 (13.9)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 8.3	0.0, 20.8
Min, Max	-25, 50	-17, 25
Week 52		
n	16	12
Mean (SD)	80.7 (17.4)	88.9 (15.2)
Median	83.3	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.6.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 95.8	79.2, 100.0
Min, Max	42, 100	58, 100
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	-2.1 (21.2)	0.0 (21.3)
Median	0.0	0.0
25th, 75th Percentile	-20.8, 8.3	0.0, 8.3
Min, Max	-33, 42	-42, 42
Difference in change from baseline (95%CI)		2.08 (-14.59, 18.76)
P-value ^b		0.7994
Hedges'g (95% CI) ^c		0.10 (-0.65, 0.84)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.6.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Bodily Pain Score		
Baseline		
n	13	19
Mean (SD)	76.0 (16.2)	88.2 (11.2)
Median	75.0	83.3
25th, 75th Percentile	75.0, 83.3	75.0, 100.0
Min, Max	33, 100	75, 100
Week 26		
n	13	19
Mean (SD)	77.6 (17.8)	80.3 (17.8)
Median	75.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.4.6.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 91.7	66.7, 100.0
Min, Max	42, 100	42, 100
Change from baseline to Week 26 ^a		
n	12	19
Mean (SD)	1.0 (19.1)	-7.9 (17.9)
Median	0.0	-8.3
25th, 75th Percentile	-16.7, 12.5	-25.0, 8.3
Min, Max	-21, 42	-33, 25
Week 52		
n	14	18
Mean (SD)	75.6 (15.5)	83.8 (19.7)
Median	75.0	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.004.006.101_qs_sum_ovr_qol_pain_haz_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.4.6.101
Infant Toddler Quality of Life (ITQoL): Bodily Pain Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	66.7, 83.3	75.0, 100.0
Min, Max	50, 100	17, 100
Change from baseline to Week 52 ^a		
n	13	18
Mean (SD)	-2.2 (22.9)	-4.6 (22.7)
Median	0.0	0.0
25th, 75th Percentile	-16.7, 8.3	-8.3, 8.3
Min, Max	-33, 50	-83, 25
Difference in change from baseline (95%CI)		-2.39 (-19.34, 14.57)
P-value ^b		0.7756
Hedges'g (95% CI) ^c		-0.10 (-0.81, 0.61)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.7026

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.5.2.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Temperament and Mood Score		
Baseline		
n	13	17
Mean (SD)	78.9 (10.8)	79.9 (9.0)
Median	83.3	80.6
25th, 75th Percentile	72.2, 84.7	75.0, 86.1
Min, Max	61, 94	60, 92
Week 26		
n	12	16
Mean (SD)	83.7 (5.8)	74.8 (8.9)
Median	84.7	77.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.005.002.101_qs_sum_ovr_qol_mood_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.5.2.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	81.9, 88.2	66.7, 81.9
Min, Max	68, 89	60, 88
Change from baseline to Week 26 ^a		
n	12	16
Mean (SD)	3.3 (8.1)	-5.4 (10.5)
Median	3.0	-3.5
25th, 75th Percentile	-3.5, 9.8	-14.6, 2.1
Min, Max	-7, 17	-22, 15
Week 52		
n	13	15
Mean (SD)	83.7 (10.9)	79.0 (12.2)
Median	87.5	81.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.5.2.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	76.4, 90.3	76.4, 86.1
Min, Max	58, 96	47, 91
Change from baseline to Week 52 ^a		
n	13	15
Mean (SD)	4.7 (9.1)	0.2 (8.1)
Median	6.0	1.4
25th, 75th Percentile	-2.8, 11.1	-2.8, 5.6
Min, Max	-13, 17	-25, 8
Difference in change from baseline (95%CI)		-4.57 (-11.24, 2.11)
P-value ^b		0.1718
Hedges'g (95% CI) ^c		-0.52 (-1.27, 0.24)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.5.2.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Temperament and Mood Score		
Baseline		
n	18	15
Mean (SD)	83.9 (7.3)	82.8 (8.1)
Median	84.0	81.9
25th, 75th Percentile	79.2, 88.9	77.8, 90.3
Min, Max	71, 96	65, 93
Week 26		
n	16	15
Mean (SD)	82.6 (8.5)	83.4 (9.0)
Median	84.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.2.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	77.1, 87.5	77.8, 90.3
Min, Max	65, 99	67, 99
Change from baseline to Week 26 ^a		
n	15	15
Mean (SD)	-1.0 (9.4)	0.6 (6.3)
Median	-2.8	1.4
25th, 75th Percentile	-8.3, 1.4	-2.8, 5.6
Min, Max	-14, 19	-11, 11
Week 52		
n	17	15
Mean (SD)	83.6 (8.1)	83.9 (10.1)
Median	83.3	80.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.2.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.9, 88.9	76.4, 94.4
Min, Max	65, 97	67, 97
Change from baseline to Week 52 ^a		
n	16	15
Mean (SD)	-0.6 (7.8)	1.1 (6.3)
Median	-1.4	2.8
25th, 75th Percentile	-6.3, 5.6	-5.6, 5.6
Min, Max	-14, 14	-13, 8
Difference in change from baseline (95%CI)		1.70 (-3.53, 6.92)
P-value ^b		0.5116
Hedges'g (95% CI) ^c		0.23 (-0.48, 0.94)
P-value for interaction term, treatment * [Sex]		0.1316

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.2.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Temperament and Mood Score		
Baseline		
n	7	7
Mean (SD)	77.0 (7.5)	81.5 (10.2)
Median	75.0	84.7
25th, 75th Percentile	72.2, 84.7	80.6, 87.5
Min, Max	65, 85	60, 90
Week 26		
n	7	7
Mean (SD)	85.2 (3.7)	79.8 (5.1)
Median	86.1	77.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.5.2.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	81.9, 88.9	75.0, 86.1
Min, Max	79, 89	75, 88
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	8.2 (6.7)	-1.8 (9.5)
Median	5.8	-1.4
25th, 75th Percentile	4.2, 13.9	-8.3, 2.8
Min, Max	-1, 17	-15, 15
Week 52		
n	7	6
Mean (SD)	87.9 (5.4)	82.1 (8.7)
Median	90.3	84.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.5.2.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	86.1, 91.7	76.4, 88.9
Min, Max	76, 92	68, 91
Change from baseline to Week 52 ^a		
n	7	6
Mean (SD)	10.9 (6.1)	1.1 (4.7)
Median	11.1	-0.7
25th, 75th Percentile	7.0, 16.7	-1.4, 5.1
Min, Max	1, 17	-4, 8
Difference in change from baseline (95%CI)		-9.84 (-16.56, -3.12)
P-value ^b		0.0081
Hedges'g (95% CI) ^c		-1.67 (-2.93, -0.35)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.2.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Temperament and Mood Score		
Baseline		
n	8	8
Mean (SD)	88.0 (5.6)	83.2 (9.0)
Median	87.5	85.4
25th, 75th Percentile	83.3, 93.1	78.5, 90.3
Min, Max	81, 96	65, 92
Week 26		
n	7	8
Mean (SD)	83.7 (7.0)	83.7 (11.6)
Median	83.3	85.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.2.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	79.2, 90.3	74.3, 92.4
Min, Max	74, 94	67, 99
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	-4.2 (8.2)	0.5 (5.3)
Median	-4.9	1.4
25th, 75th Percentile	-9.7, -1.4	-0.7, 3.5
Min, Max	-14, 10	-11, 7
Week 52		
n	8	8
Mean (SD)	86.8 (5.8)	83.9 (11.8)
Median	85.4	85.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.2.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	81.9, 91.0	73.6, 95.1
Min, Max	81, 97	67, 96
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	-1.3 (8.7)	0.7 (5.1)
Median	-2.8	2.1
25th, 75th Percentile	-8.3, 8.3	-2.8, 4.9
Min, Max	-14, 10	-8, 6
Difference in change from baseline (95%CI)		2.04 (-5.79, 9.86)
P-value ^b		0.5835
Hedges'g (95% CI) ^c		0.27 (-0.75, 1.29)
P-value for interaction term, treatment * [Sex]		0.0214

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Temperament and Mood Score		
Baseline		
n	24	21
Mean (SD)	83.8 (7.6)	81.7 (9.1)
Median	84.0	81.9
25th, 75th Percentile	79.2, 89.6	77.8, 88.9
Min, Max	66, 96	60, 93
Week 26		
n	22	20
Mean (SD)	84.3 (6.9)	80.6 (9.3)
Median	84.7	81.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.6, 88.9	75.7, 86.8
Min, Max	68, 99	60, 99
Change from baseline to Week 26 ^a		
n	21	20
Mean (SD)	0.8 (7.8)	-1.4 (9.1)
Median	-1.4	0.7
25th, 75th Percentile	-4.2, 4.2	-7.6, 4.2
Min, Max	-10, 19	-21, 15
Week 52		
n	23	19
Mean (SD)	85.5 (8.0)	83.9 (9.8)
Median	87.5	86.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.005.003.101_qs_sum_ovr_qol_mood_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.5.3.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	81.9, 91.7	79.2, 91.2
Min, Max	65, 97	58, 97
Change from baseline to Week 52 ^a		
n	22	19
Mean (SD)	1.5 (8.7)	2.9 (5.4)
Median	1.4	5.1
25th, 75th Percentile	-4.2, 7.0	1.4, 6.9
Min, Max	-14, 17	-13, 8
Difference in change from baseline (95%CI)		1.43 (-3.10, 5.95)
P-value ^b		0.5268
Hedges'g (95% CI) ^c		0.19 (-0.43, 0.80)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.5.3.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Temperament and Mood Score		
Baseline		
n	7	11
Mean (SD)	74.8 (10.8)	80.4 (7.8)
Median	73.6	80.6
25th, 75th Percentile	65.3, 87.5	73.6, 87.5
Min, Max	61, 90	65, 90
Week 26		
n	6	11
Mean (SD)	78.5 (7.8)	75.9 (10.4)
Median	80.7	77.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	73.6, 84.7	66.7, 81.9
Min, Max	65, 86	61, 94
Change from baseline to Week 26 ^a		
n	6	11
Mean (SD)	1.4 (13.0)	-4.5 (9.3)
Median	0.1	-1.4
25th, 75th Percentile	-9.7, 15.3	-11.1, 2.8
Min, Max	-14, 17	-22, 8
Week 52		
n	7	11
Mean (SD)	77.6 (11.0)	77.1 (12.9)
Median	79.2	79.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	69.4, 87.5	70.8, 86.1
Min, Max	58, 90	47, 96
Change from baseline to Week 52 ^a		
n	7	11
Mean (SD)	2.8 (9.1)	-3.3 (8.4)
Median	-2.8	-1.4
25th, 75th Percentile	-5.6, 11.1	-5.6, 1.4
Min, Max	-6, 17	-25, 6
Difference in change from baseline (95%CI)		-6.06 (-14.91, 2.79)
P-value ^b		0.1660
Hedges'g (95% CI) ^c		-0.67 (-1.63, 0.32)
P-value for interaction term, treatment * [Ethnicity]		0.0996

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Temperament and Mood Score		
Baseline		
n	12	8
Mean (SD)	84.7 (7.1)	82.6 (10.3)
Median	84.7	85.4
25th, 75th Percentile	81.3, 89.6	79.9, 89.6
Min, Max	72, 96	60, 92
Week 26		
n	11	8
Mean (SD)	86.1 (4.5)	85.1 (7.6)
Median	86.1	85.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.5.3.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	83.3, 88.9	79.2, 88.9
Min, Max	79, 94	75, 99
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	2.4 (8.3)	2.4 (6.8)
Median	1.4	2.1
25th, 75th Percentile	-2.8, 9.7	-0.7, 4.9
Min, Max	-10, 14	-8, 15
Week 52		
n	12	7
Mean (SD)	88.5 (4.7)	85.2 (10.4)
Median	89.6	90.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	84.7, 91.7	76.4, 94.4
Min, Max	81, 97	68, 96
Change from baseline to Week 52 ^a		
n	11	7
Mean (SD)	4.1 (9.5)	2.9 (4.5)
Median	7.0	4.2
25th, 75th Percentile	-2.8, 9.7	0.0, 5.6
Min, Max	-14, 17	-6, 8
Difference in change from baseline (95%CI)		-1.16 (-9.38, 7.05)
P-value ^b		0.7676
Hedges'g (95% CI) ^c		-0.14 (-1.09, 0.81)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Temperament and Mood Score		
Baseline		
n	3	7
Mean (SD)	75.5 (11.2)	82.1 (8.7)
Median	73.6	81.9
25th, 75th Percentile	65.3, 87.5	79.2, 90.3
Min, Max	65, 88	65, 90
Week 26		
n	3	7
Mean (SD)	78.3 (4.3)	78.2 (9.8)
Median	79.4	77.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	73.6, 81.9	68.1, 86.1
Min, Max	74, 82	67, 94
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	2.9 (15.5)	-4.0 (6.9)
Median	5.8	-1.4
25th, 75th Percentile	-13.9, 16.7	-11.1, 1.4
Min, Max	-14, 17	-15, 4
Week 52		
n	3	7
Mean (SD)	82.9 (7.0)	81.0 (10.3)
Median	81.9	81.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.3.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	76.4, 90.3	70.8, 88.9
Min, Max	76, 90	67, 96
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	7.4 (11.6)	-1.2 (4.4)
Median	11.1	-1.4
25th, 75th Percentile	-5.6, 16.7	-4.2, 1.4
Min, Max	-6, 17	-8, 6
Difference in change from baseline (95%CI)		-8.60 (-19.58, 2.39)
P-value ^b		0.1087
Hedges'g (95% CI) ^c		-1.13 (-2.55, 0.36)
P-value for interaction term, treatment * [Ethnicity]		0.2608

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Temperament and Mood Score		
Baseline		
n	3	8
Mean (SD)	73.6 (9.1)	81.9 (7.9)
Median	72.2	81.9
25th, 75th Percentile	65.3, 83.3	79.9, 88.2
Min, Max	65, 83	65, 90
Week 26		
n	4	8
Mean (SD)	87.5 (5.2)	78.0 (7.8)
Median	86.8	78.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	84.0, 91.0	71.5, 84.7
Min, Max	82, 94	67, 88
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	11.6 (6.6)	-4.0 (6.1)
Median	13.9	-1.4
25th, 75th Percentile	4.2, 16.7	-7.6, 0.0
Min, Max	4, 17	-15, 1
Week 52		
n	4	8
Mean (SD)	86.8 (7.0)	80.2 (9.4)
Median	89.6	79.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	82.6, 91.0	73.6, 87.5
Min, Max	76, 92	67, 94
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	11.6 (4.9)	-1.7 (4.3)
Median	11.1	-1.4
25th, 75th Percentile	7.0, 16.7	-4.9, 0.7
Min, Max	7, 17	-8, 6
Difference in change from baseline (95%CI)		-13.31 (-20.12, -6.51)
P-value ^b		0.0017
Hedges'g (95% CI) ^c		-2.74 (-4.52, -0.88)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Temperament and Mood Score		
Baseline		
n	12	7
Mean (SD)	85.2 (6.9)	82.9 (11.3)
Median	84.7	86.1
25th, 75th Percentile	81.3, 89.6	77.8, 90.3
Min, Max	74, 96	60, 92
Week 26		
n	10	7
Mean (SD)	83.2 (5.2)	86.3 (8.8)
Median	83.3	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.5.4.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	79.4, 88.9	77.8, 94.4
Min, Max	74, 90	75, 99
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-0.3 (8.7)	3.4 (7.1)
Median	-1.4	2.8
25th, 75th Percentile	-6.9, 5.8	0.0, 6.9
Min, Max	-14, 14	-8, 15
Week 52		
n	11	6
Mean (SD)	87.5 (5.2)	87.0 (10.8)
Median	87.5	90.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	81.9, 91.7	80.6, 95.8
Min, Max	81, 97	68, 96
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	2.9 (9.9)	4.3 (2.8)
Median	2.8	4.6
25th, 75th Percentile	-5.6, 9.7	2.8, 5.6
Min, Max	-14, 17	0, 8
Difference in change from baseline (95%CI)		1.38 (-5.53, 8.29)
P-value ^b		0.6721
Hedges'g (95% CI) ^c		0.16 (-0.84, 1.15)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.0213

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Temperament and Mood Score		
Baseline		
n	3	8
Mean (SD)	73.6 (9.1)	81.9 (7.9)
Median	72.2	81.9
25th, 75th Percentile	65.3, 83.3	79.9, 88.2
Min, Max	65, 83	65, 90
Week 26		
n	4	8
Mean (SD)	87.5 (5.2)	78.0 (7.8)
Median	86.8	78.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	84.0, 91.0	71.5, 84.7
Min, Max	82, 94	67, 88
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	11.6 (6.6)	-4.0 (6.1)
Median	13.9	-1.4
25th, 75th Percentile	4.2, 16.7	-7.6, 0.0
Min, Max	4, 17	-15, 1
Week 52		
n	4	8
Mean (SD)	86.8 (7.0)	80.2 (9.4)
Median	89.6	79.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.4.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	82.6, 91.0	73.6, 87.5
Min, Max	76, 92	67, 94
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	11.6 (4.9)	-1.7 (4.3)
Median	11.1	-1.4
25th, 75th Percentile	7.0, 16.7	-4.9, 0.7
Min, Max	7, 17	-8, 6
Difference in change from baseline (95%CI)		-13.31 (-20.12, -6.51)
P-value ^b		0.0017
Hedges'g (95% CI) ^c		-2.74 (-4.52, -0.88)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.4.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Temperament and Mood Score		
Baseline		
n	12	7
Mean (SD)	85.2 (6.9)	82.9 (11.3)
Median	84.7	86.1
25th, 75th Percentile	81.3, 89.6	77.8, 90.3
Min, Max	74, 96	60, 92
Week 26		
n	10	7
Mean (SD)	83.2 (5.2)	86.3 (8.8)
Median	83.3	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.4.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	79.4, 88.9	77.8, 94.4
Min, Max	74, 90	75, 99
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-0.3 (8.7)	3.4 (7.1)
Median	-1.4	2.8
25th, 75th Percentile	-6.9, 5.8	0.0, 6.9
Min, Max	-14, 14	-8, 15
Week 52		
n	11	6
Mean (SD)	87.5 (5.2)	87.0 (10.8)
Median	87.5	90.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

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Table 14.2.13.5.4.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	81.9, 91.7	80.6, 95.8
Min, Max	81, 97	68, 96
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	2.9 (9.9)	4.3 (2.8)
Median	2.8	4.6
25th, 75th Percentile	-5.6, 9.7	2.8, 5.6
Min, Max	-14, 17	0, 8
Difference in change from baseline (95%CI)		1.38 (-5.53, 8.29)
P-value ^b		0.6721
Hedges'g (95% CI) ^c		0.16 (-0.84, 1.15)
P-value for interaction term, treatment *[Cohort 1 Age Stratum]		0.0213

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

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Table 14.2.13.5.5.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Temperament and Mood Score		
Baseline		
n	11	7
Mean (SD)	85.1 (7.5)	81.5 (11.8)
Median	84.7	84.7
25th, 75th Percentile	80.6, 94.4	72.2, 90.3
Min, Max	74, 96	60, 92
Week 26		
n	11	7
Mean (SD)	85.5 (4.9)	81.3 (13.0)
Median	84.7	77.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.5.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.6, 88.9	75.0, 94.4
Min, Max	79, 94	61, 99
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.6 (7.6)	-0.2 (10.7)
Median	-1.4	2.8
25th, 75th Percentile	-5.6, 5.8	-11.1, 6.9
Min, Max	-10, 14	-15, 15
Week 52		
n	12	6
Mean (SD)	89.4 (5.1)	79.6 (19.0)
Median	90.3	85.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.5.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.4, 91.7	68.1, 95.8
Min, Max	81, 97	47, 96
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	4.1 (9.5)	-1.4 (12.1)
Median	7.0	2.1
25th, 75th Percentile	-2.8, 9.7	-1.4, 5.6
Min, Max	-14, 17	-25, 8
Difference in change from baseline (95%CI)		-5.46 (-16.76, 5.84)
P-value ^b		0.3194
Hedges'g (95% CI) ^c		-0.50 (-1.50, 0.52)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.5.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Temperament and Mood Score		
Baseline		
n	20	25
Mean (SD)	80.0 (9.5)	81.2 (7.7)
Median	81.3	80.6
25th, 75th Percentile	73.6, 88.9	77.8, 87.5
Min, Max	61, 92	63, 93
Week 26		
n	17	24
Mean (SD)	81.5 (8.4)	78.3 (8.9)
Median	84.7	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.5.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 86.1	71.5, 84.7
Min, Max	65, 99	60, 92
Change from baseline to Week 26 ^a		
n	17	24
Mean (SD)	1.1 (9.8)	-3.2 (8.8)
Median	-1.4	-1.4
25th, 75th Percentile	-5.6, 4.2	-9.5, 2.8
Min, Max	-14, 19	-22, 11
Week 52		
n	18	24
Mean (SD)	79.8 (9.4)	81.9 (9.1)
Median	82.6	81.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.5.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.2, 87.5	76.4, 88.2
Min, Max	58, 93	58, 97
Change from baseline to Week 52 ^a		
n	18	24
Mean (SD)	0.4 (8.1)	1.2 (5.6)
Median	-1.4	2.1
25th, 75th Percentile	-5.6, 6.0	-3.5, 5.6
Min, Max	-13, 17	-13, 8
Difference in change from baseline (95%CI)		0.74 (-3.52, 5.00)
P-value ^b		0.7281
Hedges'g (95% CI) ^c		0.11 (-0.51, 0.72)
P-value for interaction term, treatment * [Baseline AGV Category]		0.1952

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.5.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
ITQoL : Temperament and Mood Score		
Baseline		
n	10	6
Mean (SD)	84.2 (7.2)	83.1 (12.1)
Median	84.0	87.5
25th, 75th Percentile	80.6, 87.5	81.9, 90.3
Min, Max	74, 96	60, 92
Week 26		
n	10	6
Mean (SD)	85.2 (5.0)	84.7 (10.3)
Median	84.0	82.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.005.005.102_qs_sum_ovr_qol_mood_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.5.5.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	80.6, 88.9	75.0, 94.4
Min, Max	79, 94	75, 99
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	1.3 (7.7)	1.6 (10.4)
Median	-1.4	3.5
25th, 75th Percentile	-2.8, 5.8	-4.2, 6.9
Min, Max	-10, 14	-15, 15
Week 52		
n	11	5
Mean (SD)	88.8 (4.9)	86.1 (11.6)
Median	90.3	88.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.5.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	83.3, 91.7	81.9, 95.8
Min, Max	81, 97	68, 96
Change from baseline to Week 52 ^a		
n	10	5
Mean (SD)	4.3 (10.0)	3.3 (4.0)
Median	7.0	4.2
25th, 75th Percentile	-2.8, 9.7	0.0, 5.6
Min, Max	-14, 17	-1, 8
Difference in change from baseline (95%CI)		-1.01 (-11.18, 9.16)
P-value ^b		0.8338
Hedges'g (95% CI) ^c		-0.11 (-1.18, 0.97)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.5.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Temperament and Mood Score		
Baseline		
n	5	9
Mean (SD)	80.3 (11.1)	81.9 (7.7)
Median	84.7	81.9
25th, 75th Percentile	72.2, 87.5	79.2, 87.5
Min, Max	65, 92	65, 90
Week 26		
n	4	9
Mean (SD)	82.6 (6.7)	79.9 (8.2)
Median	84.0	80.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.5.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	77.8, 87.5	77.8, 86.1
Min, Max	74, 89	67, 90
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	5.2 (13.8)	-2.0 (4.7)
Median	9.0	-1.4
25th, 75th Percentile	-4.9, 15.3	-1.4, 1.4
Min, Max	-14, 17	-11, 3
Week 52		
n	4	9
Mean (SD)	83.3 (5.4)	81.4 (9.7)
Median	84.0	80.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.5.102
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	79.2, 87.5	76.4, 90.3
Min, Max	76, 89	67, 94
Change from baseline to Week 52 ^a		
n	4	9
Mean (SD)	5.9 (9.9)	-0.5 (4.8)
Median	6.3	0.0
25th, 75th Percentile	-2.1, 13.9	-4.2, 2.8
Min, Max	-6, 17	-8, 6
Difference in change from baseline (95%CI)		-6.42 (-15.14, 2.30)
P-value ^b		0.1334
Hedges'g (95% CI) ^c		-0.91 (-2.12, 0.35)
P-value for interaction term, treatment * [Baseline AGV Category]		0.3985

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.6.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Temperament and Mood Score		
Baseline		
n	18	13
Mean (SD)	82.5 (9.4)	81.5 (9.8)
Median	83.3	84.7
25th, 75th Percentile	75.0, 90.3	79.2, 90.3
Min, Max	65, 96	60, 92
Week 26		
n	15	12
Mean (SD)	83.4 (7.2)	79.4 (11.3)
Median	83.3	77.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.6.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	79.4, 88.9	71.5, 88.9
Min, Max	68, 99	63, 99
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	2.3 (10.4)	-2.7 (10.3)
Median	-1.4	-0.7
25th, 75th Percentile	-5.6, 13.9	-9.7, 3.5
Min, Max	-14, 19	-22, 15
Week 52		
n	16	12
Mean (SD)	86.9 (6.9)	82.4 (10.3)
Median	88.2	81.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.6.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	81.9, 91.7	73.6, 90.7
Min, Max	72, 97	67, 96
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	4.8 (9.4)	1.1 (4.9)
Median	6.5	0.7
25th, 75th Percentile	-2.8, 12.5	-2.1, 5.3
Min, Max	-14, 17	-8, 8
Difference in change from baseline (95%CI)		-3.68 (-9.36, 1.99)
P-value ^b		0.1927
Hedges'g (95% CI) ^c		-0.46 (-1.21, 0.31)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.6.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Temperament and Mood Score		
Baseline		
n	13	19
Mean (SD)	80.9 (9.0)	81.1 (7.8)
Median	83.3	80.6
25th, 75th Percentile	75.0, 88.9	75.0, 87.5
Min, Max	61, 92	63, 93
Week 26		
n	13	19
Mean (SD)	82.7 (7.8)	78.7 (9.0)
Median	84.7	81.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.5.6.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	80.6, 87.5	75.0, 86.1
Min, Max	65, 94	60, 92
Change from baseline to Week 26 ^a		
n	12	19
Mean (SD)	-0.8 (6.8)	-2.4 (8.6)
Median	-1.4	-1.4
25th, 75th Percentile	-5.6, 2.8	-10.7, 2.8
Min, Max	-10, 15	-21, 11
Week 52		
n	14	18
Mean (SD)	79.9 (10.2)	80.8 (12.2)
Median	83.3	81.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.5.6.101
Infant Toddler Quality of Life (ITQoL): Temperament and Mood Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 87.5	76.4, 87.5
Min, Max	58, 92	47, 97
Change from baseline to Week 52 ^a		
n	13	18
Mean (SD)	-1.9 (6.1)	0.3 (8.5)
Median	-2.8	2.8
25th, 75th Percentile	-5.6, 1.4	-4.2, 5.6
Min, Max	-13, 8	-25, 8
Difference in change from baseline (95%CI)		2.23 (-3.40, 7.86)
P-value ^b		0.4243
Hedges'g (95% CI) ^c		0.29 (-0.43, 1.00)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.1508

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Behavior Score		
Baseline		
n	11	11
Mean (SD)	71.9 (16.5)	78.2 (13.6)
Median	75.0	81.3
25th, 75th Percentile	56.3, 83.3	64.6, 85.4
Min, Max	40, 95	58, 100
Week 26		
n	12	12
Mean (SD)	81.8 (7.9)	72.7 (11.1)
Median	82.3	72.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 86.5	62.5, 79.2
Min, Max	71, 96	58, 94
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	9.0 (12.9)	-7.4 (8.2)
Median	4.2	-6.3
25th, 75th Percentile	0.0, 12.5	-10.4, -4.2
Min, Max	-2, 35	-25, 6
Week 52		
n	12	15
Mean (SD)	77.6 (16.2)	81.9 (13.9)
Median	78.1	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	65.6, 92.7	77.1, 93.8
Min, Max	48, 100	54, 98
Change from baseline to Week 52 ^a		
n	10	10
Mean (SD)	5.5 (20.2)	-1.2 (6.5)
Median	1.2	-2.1
25th, 75th Percentile	-8.3, 18.8	-4.2, 2.1
Min, Max	-17, 52	-10, 13
Difference in change from baseline (95%CI)		-6.71 (-21.48, 8.07)
P-value ^b		0.3390
Hedges'g (95% CI) ^c		-0.43 (-1.31, 0.46)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Behavior Score		
Baseline		
n	9	9
Mean (SD)	87.0 (9.5)	83.8 (11.6)
Median	85.4	85.4
25th, 75th Percentile	79.2, 95.8	75.0, 89.6
Min, Max	75, 100	67, 100
Week 26		
n	10	12
Mean (SD)	78.9 (13.3)	81.9 (13.2)
Median	81.3	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.006.002.101_qs_sum_ovr_qol_beha_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.2.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.5, 91.7	71.9, 94.8
Min, Max	56, 94	60, 100
Change from baseline to Week 26 ^a		
n	7	9
Mean (SD)	-4.5 (15.8)	-1.6 (7.2)
Median	-4.6	-2.1
25th, 75th Percentile	-14.6, 8.3	-6.3, 0.0
Min, Max	-33, 13	-10, 10
Week 52		
n	17	15
Mean (SD)	83.5 (17.4)	82.4 (11.9)
Median	89.6	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.2.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 93.8	70.8, 93.8
Min, Max	38, 100	65, 98
Change from baseline to Week 52 ^a		
n	8	9
Mean (SD)	-1.3 (22.1)	0.9 (11.8)
Median	4.2	2.1
25th, 75th Percentile	-7.3, 13.5	-8.3, 10.4
Min, Max	-50, 19	-19, 19
Difference in change from baseline (95%CI)		2.23 (-15.79, 20.25)
P-value ^b		0.7958
Hedges'g (95% CI) ^c		0.12 (-0.83, 1.07)
P-value for interaction term, treatment * [Sex]		0.4078

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.2.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Behavior Score		
Baseline		
n	7	7
Mean (SD)	69.0 (16.0)	83.9 (11.6)
Median	70.8	81.3
25th, 75th Percentile	56.3, 81.3	77.1, 97.9
Min, Max	40, 83	67, 100
Week 26		
n	7	7
Mean (SD)	80.4 (7.2)	75.3 (8.4)
Median	81.3	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.6.2.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 85.4	72.9, 81.3
Min, Max	71, 92	60, 88
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	11.3 (14.4)	-8.6 (7.9)
Median	4.2	-6.3
25th, 75th Percentile	0.0, 27.1	-10.4, -4.2
Min, Max	-2, 35	-25, 0
Week 52		
n	6	6
Mean (SD)	80.6 (13.9)	87.2 (8.0)
Median	79.2	86.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 91.7	81.3, 93.8
Min, Max	63, 100	77, 98
Change from baseline to Week 52 ^a		
n	6	6
Mean (SD)	9.4 (23.6)	0.3 (6.9)
Median	3.1	-1.0
25th, 75th Percentile	-8.3, 18.8	-2.1, 2.1
Min, Max	-13, 52	-8, 13
Difference in change from baseline (95%CI)		-9.03 (-33.75, 15.69)
P-value ^b		0.4039
Hedges'g (95% CI) ^c		-0.48 (-1.62, 0.68)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Behavior Score		
Baseline		
n	8	8
Mean (SD)	87.2 (10.1)	83.6 (12.4)
Median	84.4	82.3
25th, 75th Percentile	79.2, 97.9	74.0, 94.8
Min, Max	75, 100	67, 100
Week 26		
n	7	8
Mean (SD)	80.3 (11.8)	80.5 (15.5)
Median	85.4	79.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.5, 91.7	66.7, 95.8
Min, Max	63, 94	60, 100
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	-6.7 (16.2)	-3.1 (6.0)
Median	-5.4	-4.2
25th, 75th Percentile	-14.6, 6.3	-7.3, 0.0
Min, Max	-33, 13	-10, 8
Week 52		
n	8	8
Mean (SD)	83.3 (20.0)	83.3 (10.7)
Median	90.6	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.2.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	81.3, 93.8	72.9, 92.7
Min, Max	38, 98	71, 98
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	-3.0 (23.3)	-0.3 (12.0)
Median	-2.1	0.0
25th, 75th Percentile	-8.3, 14.6	-8.3, 7.3
Min, Max	-50, 19	-19, 19
Difference in change from baseline (95%CI)		2.71 (-17.58, 23.01)
P-value ^b		0.7771
Hedges'g (95% CI) ^c		0.14 (-0.88, 1.15)
P-value for interaction term, treatment * [Sex]		0.4041

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Behavior Score		
Baseline		
n	15	11
Mean (SD)	79.3 (15.7)	80.5 (11.3)
Median	81.3	83.3
25th, 75th Percentile	75.0, 87.5	75.0, 85.4
Min, Max	40, 100	58, 100
Week 26		
n	17	15
Mean (SD)	82.5 (8.5)	79.7 (11.7)
Median	83.3	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.3.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 87.5	68.8, 93.8
Min, Max	70, 96	60, 98
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	6.1 (13.6)	-2.5 (8.2)
Median	4.2	-4.2
25th, 75th Percentile	-2.1, 12.5	-6.3, 6.3
Min, Max	-15, 35	-17, 10
Week 52		
n	22	19
Mean (SD)	82.8 (15.3)	82.8 (12.1)
Median	88.5	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.9, 93.8	72.9, 93.8
Min, Max	38, 100	54, 96
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	3.7 (23.3)	-1.5 (10.4)
Median	2.1	-3.1
25th, 75th Percentile	-8.3, 14.6	-8.3, 2.1
Min, Max	-50, 52	-19, 19
Difference in change from baseline (95%CI)		-5.17 (-20.44, 10.10)
P-value ^b		0.4849
Hedges'g (95% CI) ^c		-0.26 (-1.09, 0.57)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Behavior Score		
Baseline		
n	5	9
Mean (SD)	77.1 (16.6)	81.0 (15.1)
Median	81.3	81.3
25th, 75th Percentile	70.8, 85.4	66.7, 97.9
Min, Max	52, 96	65, 100
Week 26		
n	5	9
Mean (SD)	73.8 (14.6)	73.4 (14.2)
Median	75.0	72.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	62.5, 83.3	60.4, 81.3
Min, Max	56, 92	58, 100
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	-5.2 (19.4)	-7.6 (7.4)
Median	1.0	-6.3
25th, 75th Percentile	-17.7, 7.3	-8.3, -4.2
Min, Max	-33, 10	-25, 0
Week 52		
n	7	11
Mean (SD)	75.6 (21.4)	81.1 (14.3)
Median	75.0	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	50.0, 97.9	68.8, 93.8
Min, Max	48, 100	54, 98
Change from baseline to Week 52 ^a		
n	5	9
Mean (SD)	-0.8 (13.2)	1.2 (8.0)
Median	-4.2	2.1
25th, 75th Percentile	-6.3, 4.2	-2.1, 4.2
Min, Max	-17, 19	-10, 13
Difference in change from baseline (95%CI)		1.99 (-10.23, 14.21)
P-value ^b		0.7289
Hedges'g (95% CI) ^c		0.19 (-0.91, 1.28)
P-value for interaction term, treatment * [Ethnicity]		0.5321

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Behavior Score		
Baseline		
n	12	8
Mean (SD)	77.8 (16.8)	82.0 (10.1)
Median	80.2	81.3
25th, 75th Percentile	72.9, 85.4	76.0, 87.5
Min, Max	40, 100	67, 100
Week 26		
n	11	8
Mean (SD)	81.4 (8.1)	78.6 (12.5)
Median	83.3	76.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.006.003.102_qs_sum_ovr_qol_beha_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.3.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.9, 85.4	70.8, 88.5
Min, Max	70, 94	60, 98
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	5.8 (15.4)	-3.4 (5.7)
Median	2.1	-5.2
25th, 75th Percentile	-4.6, 12.5	-6.3, -1.0
Min, Max	-15, 35	-10, 8
Week 52		
n	11	7
Mean (SD)	80.5 (18.2)	81.8 (9.5)
Median	89.6	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 93.8	70.8, 91.7
Min, Max	38, 98	71, 94
Change from baseline to Week 52 ^a		
n	10	7
Mean (SD)	1.9 (26.3)	-2.4 (11.6)
Median	0.0	-2.1
25th, 75th Percentile	-8.3, 14.6	-8.3, 2.1
Min, Max	-50, 52	-19, 19
Difference in change from baseline (95%CI)		-4.26 (-26.97, 18.46)
P-value ^b		0.6951
Hedges'g (95% CI) ^c		-0.19 (-1.15, 0.78)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.3.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Behavior Score		
Baseline		
n	3	7
Mean (SD)	82.6 (12.6)	85.7 (13.7)
Median	81.3	81.3
25th, 75th Percentile	70.8, 95.8	72.9, 100.0
Min, Max	71, 96	67, 100
Week 26		
n	3	7
Mean (SD)	76.4 (14.6)	77.4 (13.6)
Median	75.0	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	62.5, 91.7	64.6, 87.5
Min, Max	63, 92	60, 100
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	-6.2 (23.7)	-8.3 (8.4)
Median	4.2	-8.3
25th, 75th Percentile	-33.3, 10.4	-10.4, 0.0
Min, Max	-33, 10	-25, 0
Week 52		
n	3	7
Mean (SD)	88.2 (12.6)	88.1 (9.1)
Median	89.6	89.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.3.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 100.0	79.2, 97.9
Min, Max	75, 100	75, 98
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	5.6 (12.6)	2.4 (7.7)
Median	4.2	2.1
25th, 75th Percentile	-6.3, 18.8	-2.1, 12.5
Min, Max	-6, 19	-8, 13
Difference in change from baseline (95%CI)		-3.18 (-17.79, 11.44)
P-value ^b		0.6298
Hedges'g (95% CI) ^c		-0.31 (-1.66, 1.06)
P-value for interaction term, treatment * [Ethnicity]		0.9450

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Behavior Score		
Baseline		
n	3	8
Mean (SD)	64.6 (22.5)	83.1 (11.9)
Median	70.8	81.3
25th, 75th Percentile	39.6, 83.3	74.0, 93.8
Min, Max	40, 83	67, 100
Week 26		
n	4	8
Mean (SD)	79.2 (5.1)	76.8 (12.1)
Median	78.1	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 83.3	68.8, 84.4
Min, Max	75, 85	60, 98
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	12.5 (20.1)	-6.3 (9.8)
Median	4.2	-7.3
25th, 75th Percentile	-2.1, 35.4	-9.4, 0.0
Min, Max	-2, 35	-25, 8
Week 52		
n	4	8
Mean (SD)	81.8 (10.4)	85.4 (9.8)
Median	82.3	86.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.9, 90.6	77.1, 93.8
Min, Max	71, 92	71, 98
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	14.6 (33.5)	2.3 (12.3)
Median	4.2	2.1
25th, 75th Percentile	-12.5, 52.1	-5.2, 12.5
Min, Max	-13, 52	-19, 19
Difference in change from baseline (95%CI)		-12.24 (-90.45, 65.97)
P-value ^b		0.5949
Hedges'g (95% CI) ^c		-0.58 (-1.92, 0.78)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Behavior Score		
Baseline		
n	12	7
Mean (SD)	82.3 (12.5)	84.5 (12.1)
Median	81.3	83.3
25th, 75th Percentile	77.1, 91.7	77.1, 100.0
Min, Max	56, 100	67, 100
Week 26		
n	10	7
Mean (SD)	80.8 (10.8)	79.5 (13.9)
Median	84.4	77.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 91.7	68.8, 93.8
Min, Max	63, 94	60, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.2 (16.5)	-5.1 (3.4)
Median	2.1	-6.3
25th, 75th Percentile	-6.3, 10.4	-6.3, -2.1
Min, Max	-33, 27	-10, 0
Week 52		
n	10	6
Mean (SD)	82.3 (19.6)	84.4 (9.9)
Median	90.6	84.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.9, 93.8	77.1, 91.7
Min, Max	38, 100	71, 98
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-0.8 (20.4)	-3.1 (4.3)
Median	0.0	-2.1
25th, 75th Percentile	-8.3, 14.6	-8.3, 0.0
Min, Max	-50, 19	-8, 2
Difference in change from baseline (95%CI)		-2.29 (-17.15, 12.56)
P-value ^b		0.7389
Hedges'g (95% CI) ^c		-0.13 (-1.14, 0.88)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.5139

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Behavior Score		
Baseline		
n	3	8
Mean (SD)	64.6 (22.5)	83.1 (11.9)
Median	70.8	81.3
25th, 75th Percentile	39.6, 83.3	74.0, 93.8
Min, Max	40, 83	67, 100
Week 26		
n	4	8
Mean (SD)	79.2 (5.1)	76.8 (12.1)
Median	78.1	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 83.3	68.8, 84.4
Min, Max	75, 85	60, 98
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	12.5 (20.1)	-6.3 (9.8)
Median	4.2	-7.3
25th, 75th Percentile	-2.1, 35.4	-9.4, 0.0
Min, Max	-2, 35	-25, 8
Week 52		
n	4	8
Mean (SD)	81.8 (10.4)	85.4 (9.8)
Median	82.3	86.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.9, 90.6	77.1, 93.8
Min, Max	71, 92	71, 98
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	14.6 (33.5)	2.3 (12.3)
Median	4.2	2.1
25th, 75th Percentile	-12.5, 52.1	-5.2, 12.5
Min, Max	-13, 52	-19, 19
Difference in change from baseline (95%CI)		-12.24 (-90.45, 65.97)
P-value ^b		0.5949
Hedges'g (95% CI) ^c		-0.58 (-1.92, 0.78)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Behavior Score		
Baseline		
n	12	7
Mean (SD)	82.3 (12.5)	84.5 (12.1)
Median	81.3	83.3
25th, 75th Percentile	77.1, 91.7	77.1, 100.0
Min, Max	56, 100	67, 100
Week 26		
n	10	7
Mean (SD)	80.8 (10.8)	79.5 (13.9)
Median	84.4	77.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.006.004.102_qs_sum_ovr_qol_beha_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.6.4.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.8, 91.7	68.8, 93.8
Min, Max	63, 94	60, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.2 (16.5)	-5.1 (3.4)
Median	2.1	-6.3
25th, 75th Percentile	-6.3, 10.4	-6.3, -2.1
Min, Max	-33, 27	-10, 0
Week 52		
n	10	6
Mean (SD)	82.3 (19.6)	84.4 (9.9)
Median	90.6	84.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.4.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.9, 93.8	77.1, 91.7
Min, Max	38, 100	71, 98
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-0.8 (20.4)	-3.1 (4.3)
Median	0.0	-2.1
25th, 75th Percentile	-8.3, 14.6	-8.3, 0.0
Min, Max	-50, 19	-8, 2
Difference in change from baseline (95%CI)		-2.29 (-17.15, 12.56)
P-value ^b		0.7389
Hedges'g (95% CI) ^c		-0.13 (-1.14, 0.88)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.5139

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Behavior Score		
Baseline		
n	11	7
Mean (SD)	81.8 (11.3)	85.1 (15.5)
Median	81.3	83.3
25th, 75th Percentile	79.2, 87.5	66.7, 100.0
Min, Max	56, 100	65, 100
Week 26		
n	11	7
Mean (SD)	85.2 (8.1)	77.1 (15.2)
Median	85.4	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	81.3, 91.7	60.4, 93.8
Min, Max	70, 96	60, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	3.3 (11.6)	-8.0 (7.9)
Median	2.3	-6.3
25th, 75th Percentile	-4.6, 10.4	-8.3, -4.2
Min, Max	-15, 27	-25, 0
Week 52		
n	11	6
Mean (SD)	84.3 (18.2)	84.4 (16.4)
Median	91.7	87.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.9, 95.8	81.3, 97.9
Min, Max	38, 100	54, 98
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-0.6 (20.6)	-3.8 (4.6)
Median	1.2	-2.1
25th, 75th Percentile	-8.3, 14.6	-8.3, -2.1
Min, Max	-50, 19	-10, 2
Difference in change from baseline (95%CI)		-3.23 (-18.23, 11.77)
P-value ^b		0.6432
Hedges'g (95% CI) ^c		-0.18 (-1.19, 0.83)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Behavior Score		
Baseline		
n	9	13
Mean (SD)	75.0 (19.7)	78.4 (10.9)
Median	75.0	79.2
25th, 75th Percentile	70.8, 85.4	72.9, 85.4
Min, Max	40, 100	58, 98
Week 26		
n	11	17
Mean (SD)	75.8 (10.8)	77.5 (12.2)
Median	75.0	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 83.3	68.8, 83.3
Min, Max	56, 94	58, 98
Change from baseline to Week 26 ^a		
n	7	13
Mean (SD)	3.6 (20.5)	-3.0 (8.0)
Median	4.2	-4.2
25th, 75th Percentile	-2.1, 12.5	-8.3, 0.0
Min, Max	-33, 35	-17, 10
Week 52		
n	18	24
Mean (SD)	79.1 (16.2)	81.6 (12.0)
Median	82.3	82.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.6.5.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	68.8, 91.7	71.9, 93.8
Min, Max	48, 100	54, 96
Change from baseline to Week 52 ^a		
n	8	13
Mean (SD)	6.3 (21.7)	1.4 (10.4)
Median	0.0	2.1
25th, 75th Percentile	-7.3, 14.6	-4.2, 10.4
Min, Max	-17, 52	-19, 19
Difference in change from baseline (95%CI)		-4.81 (-23.31, 13.69)
P-value ^b		0.5709
Hedges'g (95% CI) ^c		-0.30 (-1.18, 0.59)
P-value for interaction term, treatment * [Baseline AGV Category]		0.8864

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
<= 4.5		
ITQoL : Behavior Score		
Baseline		
n	10	6
Mean (SD)	80.4 (10.9)	88.5 (13.8)
Median	81.3	91.7
25th, 75th Percentile	79.2, 83.3	81.3, 100.0
Min, Max	56, 100	67, 100
Week 26		
n	10	6
Mean (SD)	84.1 (7.7)	79.9 (14.5)
Median	85.4	76.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.6.5.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	81.3, 91.7	72.9, 93.8
Min, Max	70, 94	60, 100
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	3.7 (12.3)	-8.7 (8.5)
Median	4.2	-6.3
25th, 75th Percentile	-4.6, 10.4	-8.3, -6.3
Min, Max	-15, 27	-25, 0
Week 52		
n	10	5
Mean (SD)	83.1 (18.8)	90.4 (7.9)
Median	90.6	91.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.9, 93.8	83.3, 97.9
Min, Max	38, 100	81, 98
Change from baseline to Week 52 ^a		
n	9	5
Mean (SD)	-0.7 (21.8)	-2.5 (3.7)
Median	2.1	-2.1
25th, 75th Percentile	-8.3, 14.6	-2.1, -2.1
Min, Max	-50, 19	-8, 2
Difference in change from baseline (95%CI)		-1.80 (-18.72, 15.12)
P-value ^b		0.8144
Hedges'g (95% CI) ^c		-0.09 (-1.19, 1.00)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Behavior Score		
Baseline		
n	5	9
Mean (SD)	75.4 (24.2)	80.6 (9.4)
Median	70.8	79.2
25th, 75th Percentile	70.8, 95.8	75.0, 85.4
Min, Max	40, 100	67, 98
Week 26		
n	4	9
Mean (SD)	70.8 (5.9)	76.9 (11.9)
Median	72.9	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.6.5.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	66.7, 75.0	68.8, 83.3
Min, Max	63, 75	60, 98
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	1.6 (28.1)	-3.7 (6.1)
Median	2.1	-4.2
25th, 75th Percentile	-16.7, 19.8	-8.3, 0.0
Min, Max	-33, 35	-10, 8
Week 52		
n	4	9
Mean (SD)	79.7 (13.6)	81.9 (9.3)
Median	82.3	79.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.6.5.102
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	68.8, 90.6	75.0, 89.6
Min, Max	63, 92	71, 94
Change from baseline to Week 52 ^a		
n	4	9
Mean (SD)	10.4 (28.3)	1.4 (12.0)
Median	-1.0	2.1
25th, 75th Percentile	-7.3, 28.1	-8.3, 12.5
Min, Max	-8, 52	-19, 19
Difference in change from baseline (95%CI)		-9.03 (-52.35, 34.28)
P-value ^b		0.5769
Hedges'g (95% CI) ^c		-0.47 (-1.65, 0.74)
P-value for interaction term, treatment * [Baseline AGV Category]		0.6279

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.006.005.102_qs_sum_ovr_qol_beha_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.6.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Behavior Score		
Baseline		
n	17	12
Mean (SD)	80.5 (15.3)	81.6 (13.0)
Median	81.3	81.3
25th, 75th Percentile	75.0, 87.5	69.8, 92.7
Min, Max	40, 100	65, 100
Week 26		
n	15	12
Mean (SD)	83.2 (9.9)	75.0 (13.2)
Median	85.4	74.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.6.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 91.7	62.5, 82.3
Min, Max	63, 96	58, 100
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	4.0 (16.2)	-6.6 (6.5)
Median	4.2	-6.3
25th, 75th Percentile	-4.6, 12.5	-7.3, -3.1
Min, Max	-33, 35	-25, 0
Week 52		
n	15	12
Mean (SD)	85.4 (16.3)	85.8 (9.8)
Median	91.7	85.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.6.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 95.8	78.1, 94.8
Min, Max	38, 100	69, 98
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	4.6 (22.1)	1.9 (6.2)
Median	4.2	2.1
25th, 75th Percentile	-6.3, 18.8	-2.1, 4.2
Min, Max	-50, 52	-8, 13
Difference in change from baseline (95%CI)		-2.71 (-15.39, 9.96)
P-value ^b		0.6571
Hedges'g (95% CI) ^c		-0.15 (-0.93, 0.63)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.6.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Behavior Score		
Baseline		
n	3	8
Mean (SD)	68.7 (15.7)	79.4 (13.1)
Median	70.8	82.3
25th, 75th Percentile	52.1, 83.3	69.8, 87.5
Min, Max	52, 83	58, 98
Week 26		
n	7	12
Mean (SD)	74.7 (9.8)	79.7 (12.5)
Median	77.1	81.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.6.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 81.3	68.8, 90.6
Min, Max	56, 85	60, 98
Change from baseline to Week 26 ^a		
n	2	8
Mean (SD)	-1.0 (1.5)	-2.1 (10.0)
Median	-1.0	-2.1
25th, 75th Percentile	-2.1, 0.0	-10.4, 7.3
Min, Max	-2, 0	-17, 10
Week 52		
n	14	18
Mean (SD)	76.3 (16.7)	79.7 (14.1)
Median	78.1	82.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.6.6.101
Infant Toddler Quality of Life (ITQoL): Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	62.5, 89.6	70.8, 93.8
Min, Max	48, 100	54, 96
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	-8.3 (4.2)	-3.1 (12.0)
Median	-8.3	-6.3
25th, 75th Percentile	-12.5, -4.2	-9.4, 3.1
Min, Max	-13, -4	-19, 19
Difference in change from baseline (95%CI)		5.20 (-11.34, 21.75)
P-value ^b		0.4948
Hedges'g (95% CI) ^c		0.44 (-0.91, 1.77)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.5284

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Global Behavior Score		
Baseline		
n	11	11
Mean (SD)	80.9 (22.3)	85.9 (14.6)
Median	85.0	85.0
25th, 75th Percentile	60.0, 100.0	85.0, 100.0
Min, Max	30, 100	60, 100
Week 26		
n	12	12
Mean (SD)	87.9 (11.4)	82.1 (19.6)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 92.5
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	5.5 (20.1)	-4.1 (19.2)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 0.0
Min, Max	-15, 55	-55, 25
Week 52		
n	12	15
Mean (SD)	85.8 (14.0)	84.0 (24.6)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	10	10
Mean (SD)	5.5 (20.1)	-11.0 (22.5)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	-30.0, 0.0
Min, Max	-15, 55	-55, 15
Difference in change from baseline (95%CI)		-16.50 (-36.51, 3.51)
P-value ^b		0.1003
Hedges'g (95% CI) ^c		-0.74 (-1.64, 0.18)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Global Behavior Score		
Baseline		
n	9	10
Mean (SD)	93.3 (7.9)	84.5 (14.6)
Median	100.0	85.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	60, 100
Week 26		
n	10	12
Mean (SD)	86.0 (15.4)	89.6 (15.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.2.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	7	10
Mean (SD)	-1.4 (14.6)	3.0 (13.4)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 15.0	0.0, 15.0
Min, Max	-25, 15	-25, 25
Week 52		
n	17	15
Mean (SD)	88.5 (15.2)	87.0 (21.1)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	8	10
Mean (SD)	-3.1 (17.7)	4.5 (20.3)
Median	0.0	0.0
25th, 75th Percentile	-7.5, 7.5	0.0, 15.0
Min, Max	-40, 15	-25, 40
Difference in change from baseline (95%CI)		7.63 (-11.72, 26.97)
P-value ^b		0.4156
Hedges'g (95% CI) ^c		0.38 (-0.57, 1.31)
P-value for interaction term, treatment * [Sex]		0.0778

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Global Behavior Score		
Baseline		
n	7	7
Mean (SD)	77.9 (25.0)	90.0 (15.0)
Median	85.0	100.0
25th, 75th Percentile	60.0, 100.0	85.0, 100.0
Min, Max	30, 100	60, 100
Week 26		
n	7	7
Mean (SD)	85.7 (13.4)	91.4 (8.0)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	7.9 (22.5)	1.4 (11.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 0.0
Min, Max	-15, 55	-15, 25
Week 52		
n	6	6
Mean (SD)	85.8 (14.6)	90.8 (16.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	6	6
Mean (SD)	9.2 (24.4)	-4.2 (18.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 0.0
Min, Max	-15, 55	-40, 15
Difference in change from baseline (95%CI)		-13.33 (-41.20, 14.53)
P-value ^b		0.3114
Hedges'g (95% CI) ^c		-0.57 (-1.71, 0.60)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.007.002.102_qs_sum_ovr_qol_gbeha_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Global Behavior Score		
Baseline		
n	8	8
Mean (SD)	94.4 (7.8)	84.4 (16.6)
Median	100.0	85.0
25th, 75th Percentile	85.0, 100.0	72.5, 100.0
Min, Max	85, 100	60, 100
Week 26		
n	7	8
Mean (SD)	87.9 (14.4)	89.4 (14.0)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.2.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	-4.2 (13.9)	5.0 (9.6)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 7.5
Min, Max	-25, 15	0, 25
Week 52		
n	8	8
Mean (SD)	84.4 (16.6)	91.3 (14.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.2.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.5, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	-5.7 (17.4)	6.9 (19.6)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 20.0
Min, Max	-40, 15	-25, 40
Difference in change from baseline (95%CI)		12.59 (-8.26, 33.43)
P-value ^b		0.2146
Hedges'g (95% CI) ^c		0.64 (-0.42, 1.67)
P-value for interaction term, treatment * [Sex]		0.1083

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Global Behavior Score		
Baseline		
n	15	12
Mean (SD)	89.3 (18.0)	84.6 (13.2)
Median	100.0	85.0
25th, 75th Percentile	85.0, 100.0	85.0, 92.5
Min, Max	30, 100	60, 100
Week 26		
n	17	15
Mean (SD)	91.2 (7.6)	87.7 (13.3)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	60, 100
Change from baseline to Week 26 ^a		
n	13	12
Mean (SD)	5.4 (17.7)	1.3 (13.2)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 7.5
Min, Max	-15, 55	-25, 25
Week 52		
n	22	19
Mean (SD)	90.2 (12.2)	87.6 (20.4)
Median	92.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	2.3 (21.8)	-6.8 (19.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	-25.0, 15.0
Min, Max	-40, 55	-40, 15
Difference in change from baseline (95%CI)		-9.13 (-26.87, 8.62)
P-value ^b		0.2977
Hedges'g (95% CI) ^c		-0.42 (-1.23, 0.40)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Global Behavior Score		
Baseline		
n	5	9
Mean (SD)	78.0 (17.5)	86.1 (16.4)
Median	85.0	85.0
25th, 75th Percentile	60.0, 85.0	85.0, 100.0
Min, Max	60, 100	60, 100
Week 26		
n	5	9
Mean (SD)	73.0 (18.6)	82.8 (23.7)
Median	60.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 85.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	-6.3 (17.5)	-3.3 (21.1)
Median	-7.5	0.0
25th, 75th Percentile	-20.0, 7.5	0.0, 0.0
Min, Max	-25, 15	-55, 25
Week 52		
n	7	11
Mean (SD)	78.6 (18.4)	81.8 (26.6)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	5	9
Mean (SD)	0.0 (10.6)	1.1 (25.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-15, 15	-55, 40
Difference in change from baseline (95%CI)		1.11 (-25.35, 27.57)
P-value ^b		0.9286
Hedges'g (95% CI) ^c		0.05 (-1.05, 1.14)
P-value for interaction term, treatment * [Ethnicity]		0.4901

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Global Behavior Score		
Baseline		
n	12	8
Mean (SD)	89.2 (20.0)	87.5 (13.4)
Median	100.0	85.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	30, 100	60, 100
Week 26		
n	11	8
Mean (SD)	90.5 (7.6)	90.6 (7.8)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	85, 100
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	4.0 (19.8)	3.1 (11.9)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 7.5
Min, Max	-15, 55	-15, 25
Week 52		
n	11	7
Mean (SD)	85.9 (14.6)	86.4 (18.9)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	10	7
Mean (SD)	0.0 (24.3)	-5.0 (20.4)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	-25.0, 15.0
Min, Max	-40, 55	-40, 15
Difference in change from baseline (95%CI)		-5.00 (-28.95, 18.95)
P-value ^b		0.6627
Hedges'g (95% CI) ^c		-0.21 (-1.17, 0.76)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Global Behavior Score		
Baseline		
n	3	7
Mean (SD)	76.7 (14.4)	86.4 (18.9)
Median	85.0	100.0
25th, 75th Percentile	60.0, 85.0	60.0, 100.0
Min, Max	60, 85	60, 100
Week 26		
n	3	7
Mean (SD)	73.3 (23.1)	90.0 (15.0)
Median	60.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.7.3.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	-3.3 (20.2)	3.6 (9.4)
Median	0.0	0.0
25th, 75th Percentile	-25.0, 15.0	0.0, 0.0
Min, Max	-25, 15	0, 25
Week 52		
n	3	7
Mean (SD)	81.7 (20.2)	95.7 (7.3)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.3.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	5.0 (8.7)	9.3 (16.4)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 25.0
Min, Max	0, 15	0, 40
Difference in change from baseline (95%CI)		4.29 (-19.39, 27.96)
P-value ^b		0.6874
Hedges'g (95% CI) ^c		0.26 (-1.11, 1.61)
P-value for interaction term, treatment * [Ethnicity]		0.5966

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.007.003.102_qs_sum_ovr_qol_gbeha_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.4.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Global Behavior Score		
Baseline		
n	3	8
Mean (SD)	63.3 (35.1)	84.4 (16.6)
Median	60.0	85.0
25th, 75th Percentile	30.0, 100.0	72.5, 100.0
Min, Max	30, 100	60, 100
Week 26		
n	4	8
Mean (SD)	78.8 (12.5)	89.4 (14.0)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	72.5, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	13.3 (36.9)	5.0 (9.6)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 55.0	0.0, 7.5
Min, Max	-15, 55	0, 25
Week 52		
n	4	8
Mean (SD)	72.5 (14.4)	91.3 (14.3)
Median	72.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.4.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	13.3 (36.9)	6.9 (19.6)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 55.0	0.0, 20.0
Min, Max	-15, 55	-25, 40
Difference in change from baseline (95%CI)		-6.46 (-44.02, 31.10)
P-value ^b		0.7063
Hedges'g (95% CI) ^c		-0.24 (-1.57, 1.10)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.4.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Global Behavior Score		
Baseline		
n	12	7
Mean (SD)	92.5 (7.8)	90.0 (15.0)
Median	92.5	100.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	60, 100
Week 26		
n	10	7
Mean (SD)	90.0 (12.9)	91.4 (8.0)
Median	92.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-1.0 (12.0)	1.4 (11.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-25, 15	-15, 25
Week 52		
n	10	6
Mean (SD)	90.0 (12.9)	90.8 (16.3)
Median	92.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-2.5 (15.7)	-4.2 (18.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-40, 15	-40, 15
Difference in change from baseline (95%CI)		-1.67 (-20.23, 16.90)
P-value ^b		0.8501
Hedges'g (95% CI) ^c		-0.09 (-1.11, 0.92)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.7826

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
ITQoL : Global Behavior Score		
Baseline		
n	3	8
Mean (SD)	63.3 (35.1)	84.4 (16.6)
Median	60.0	85.0
25th, 75th Percentile	30.0, 100.0	72.5, 100.0
Min, Max	30, 100	60, 100
Week 26		
n	4	8
Mean (SD)	78.8 (12.5)	89.4 (14.0)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.5, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	13.3 (36.9)	5.0 (9.6)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 55.0	0.0, 7.5
Min, Max	-15, 55	0, 25
Week 52		
n	4	8
Mean (SD)	72.5 (14.4)	91.3 (14.3)
Median	72.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	13.3 (36.9)	6.9 (19.6)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 55.0	0.0, 20.0
Min, Max	-15, 55	-25, 40
Difference in change from baseline (95%CI)		-6.46 (-44.02, 31.10)
P-value ^b		0.7063
Hedges'g (95% CI) ^c		-0.24 (-1.57, 1.10)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
ITQoL : Global Behavior Score		
Baseline		
n	12	7
Mean (SD)	92.5 (7.8)	90.0 (15.0)
Median	92.5	100.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	60, 100
Week 26		
n	10	7
Mean (SD)	90.0 (12.9)	91.4 (8.0)
Median	92.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	85, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-1.0 (12.0)	1.4 (11.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-25, 15	-15, 25
Week 52		
n	10	6
Mean (SD)	90.0 (12.9)	90.8 (16.3)
Median	92.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.4.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-2.5 (15.7)	-4.2 (18.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-40, 15	-40, 15
Difference in change from baseline (95%CI)		-1.67 (-20.23, 16.90)
P-value ^b		0.8501
Hedges'g (95% CI) ^c		-0.09 (-1.11, 0.92)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.7826

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.5.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Global Behavior Score		
Baseline		
n	11	7
Mean (SD)	94.5 (7.6)	92.1 (15.2)
Median	100.0	100.0
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	60, 100
Week 26		
n	11	7
Mean (SD)	93.2 (7.8)	93.6 (8.0)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.5.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	85, 100
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.0 (10.0)	1.4 (11.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-15, 15	-15, 25
Week 52		
n	11	6
Mean (SD)	88.6 (15.7)	81.7 (29.9)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-4.0 (16.1)	-15.8 (25.0)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	-40.0, 0.0
Min, Max	-40, 15	-55, 0
Difference in change from baseline (95%CI)		-11.83 (-33.71, 10.04)
P-value ^b		0.2653
Hedges'g (95% CI) ^c		-0.57 (-1.59, 0.48)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.007.005.101_qs_sum_ovr_qol_gbeha_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Global Behavior Score		
Baseline		
n	9	14
Mean (SD)	76.7 (22.6)	81.8 (13.0)
Median	85.0	85.0
25th, 75th Percentile	60.0, 85.0	85.0, 85.0
Min, Max	30, 100	60, 100
Week 26		
n	11	17
Mean (SD)	80.9 (14.6)	82.6 (19.6)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.007.005.101_qs_sum_ovr_qol_gbeha_agv_ov_206_fas.pdf+rtf

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Table 14.2.13.7.5.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	60.0, 85.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	7	14
Mean (SD)	6.4 (25.9)	-1.8 (19.0)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 15.0	0.0, 0.0
Min, Max	-25, 55	-55, 25
Week 52		
n	18	24
Mean (SD)	86.7 (14.1)	86.5 (21.0)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	8	14
Mean (SD)	8.8 (21.0)	2.1 (19.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 15.0
Min, Max	-15, 55	-30, 40
Difference in change from baseline (95%CI)		-6.61 (-25.18, 11.97)
P-value ^b		0.4667
Hedges'g (95% CI) ^c		-0.32 (-1.19, 0.56)
P-value for interaction term, treatment * [Baseline AGV Category]		0.7027

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
ITQoL : Global Behavior Score		
Baseline		
n	10	6
Mean (SD)	94.0 (7.7)	93.3 (16.3)
Median	100.0	100.0
25th, 75th Percentile	85.0, 100.0	100.0, 100.0
Min, Max	85, 100	60, 100
Week 26		
n	10	6
Mean (SD)	92.5 (7.9)	95.0 (7.7)
Median	92.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	85, 100	85, 100
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	0.0 (10.6)	1.7 (12.9)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-15, 15	-15, 25
Week 52		
n	10	5
Mean (SD)	87.5 (16.0)	92.0 (17.9)
Median	92.5	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	85.0, 100.0	100.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	9	5
Mean (SD)	-4.4 (17.0)	-8.0 (17.9)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 0.0
Min, Max	-40, 15	-40, 0
Difference in change from baseline (95%CI)		-3.56 (-24.61, 17.50)
P-value ^b		0.7193
Hedges'g (95% CI) ^c		-0.19 (-1.28, 0.91)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.5.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Global Behavior Score		
Baseline		
n	5	9
Mean (SD)	72.0 (27.5)	82.8 (14.4)
Median	85.0	85.0
25th, 75th Percentile	60.0, 85.0	85.0, 85.0
Min, Max	30, 100	60, 100
Week 26		
n	4	9
Mean (SD)	72.5 (14.4)	87.2 (12.5)
Median	72.5	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.7.5.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	7.5 (33.8)	4.4 (9.2)
Median	0.0	0.0
25th, 75th Percentile	-12.5, 27.5	0.0, 0.0
Min, Max	-25, 55	0, 25
Week 52		
n	4	9
Mean (SD)	78.8 (12.5)	90.6 (13.6)
Median	85.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.5.102
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.5, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 52 ^a		
n	4	9
Mean (SD)	13.8 (27.5)	7.8 (18.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 27.5	0.0, 15.0
Min, Max	0, 55	-25, 40
Difference in change from baseline (95%CI)		-5.97 (-34.24, 22.29)
P-value ^b		0.6510
Hedges'g (95% CI) ^c		-0.26 (-1.44, 0.93)
P-value for interaction term, treatment * [Baseline AGV Category]		0.8804

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.6.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Global Behavior Score		
Baseline		
n	17	12
Mean (SD)	87.4 (18.3)	86.3 (17.1)
Median	85.0	92.5
25th, 75th Percentile	85.0, 100.0	72.5, 100.0
Min, Max	30, 100	60, 100
Week 26		
n	15	12
Mean (SD)	89.7 (13.9)	84.6 (20.8)
Median	100.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.6.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	4.0 (18.4)	-1.7 (20.2)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 0.0
Min, Max	-25, 55	-55, 25
Week 52		
n	15	12
Mean (SD)	89.7 (13.9)	92.9 (12.3)
Median	100.0	100.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.6.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	85.0, 100.0
Min, Max	60, 100	60, 100
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	3.0 (20.4)	3.6 (19.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 15.0	0.0, 15.0
Min, Max	-40, 55	-40, 40
Difference in change from baseline (95%CI)		0.64 (-15.87, 17.14)
P-value ^b		0.9372
Hedges'g (95% CI) ^c		0.03 (-0.75, 0.81)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.6.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Global Behavior Score		
Baseline		
n	3	9
Mean (SD)	81.7 (20.2)	83.9 (10.2)
Median	85.0	85.0
25th, 75th Percentile	60.0, 100.0	85.0, 85.0
Min, Max	60, 100	60, 100
Week 26		
n	7	12
Mean (SD)	81.4 (9.4)	87.1 (14.5)
Median	85.0	85.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.6.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 85.0	85.0, 100.0
Min, Max	60, 85	60, 100
Change from baseline to Week 26 ^a		
n	2	9
Mean (SD)	-7.5 (10.6)	0.6 (11.6)
Median	-7.5	0.0
25th, 75th Percentile	-15.0, 0.0	0.0, 0.0
Min, Max	-15, 0	-25, 15
Week 52		
n	14	18
Mean (SD)	85.0 (15.2)	80.6 (26.6)
Median	85.0	92.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.7.6.101
Infant Toddler Quality of Life (ITQoL): Global Behaviour Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	85.0, 100.0	60.0, 100.0
Min, Max	60, 100	30, 100
Change from baseline to Week 52 ^a		
n	3	9
Mean (SD)	-5.0 (8.7)	-11.7 (23.5)
Median	0.0	0.0
25th, 75th Percentile	-15.0, 0.0	-25.0, 0.0
Min, Max	-15, 0	-55, 15
Difference in change from baseline (95%CI)		-6.67 (-38.35, 25.02)
P-value ^b		0.6493
Hedges'g (95% CI) ^c		-0.29 (-1.59, 1.03)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.6491

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.007.006.101_qs_sum_ovr_qol_gbeha_haz_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Getting on With Others Score		
Baseline		
n	10	11
Mean (SD)	65.7 (18.6)	70.6 (10.9)
Median	70.8	73.3
25th, 75th Percentile	61.7, 75.0	65.0, 76.7
Min, Max	27, 87	50, 87
Week 26		
n	11	12
Mean (SD)	76.2 (9.9)	67.9 (12.1)
Median	78.3	69.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.101_qs_sum_ovr_qol_oth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 85.0	60.0, 77.5
Min, Max	55, 87	45, 83
Change from baseline to Week 26 ^a		
n	9	11
Mean (SD)	5.6 (12.7)	-3.9 (6.3)
Median	5.0	-5.0
25th, 75th Percentile	-6.7, 15.0	-6.7, 0.0
Min, Max	-8, 28	-13, 10
Week 52		
n	12	15
Mean (SD)	74.7 (15.2)	72.9 (12.2)
Median	75.8	73.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.101_qs_sum_ovr_qol_oth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 83.3	65.0, 83.3
Min, Max	37, 98	52, 95
Change from baseline to Week 52 ^a		
n	9	10
Mean (SD)	9.1 (17.9)	1.0 (10.0)
Median	3.3	0.8
25th, 75th Percentile	-3.3, 8.3	-5.0, 8.3
Min, Max	-5, 48	-13, 21
Difference in change from baseline (95%CI)		-8.11 (-21.95, 5.73)
P-value ^b		0.2331
Hedges'g (95% CI) ^c		-0.54 (-1.45, 0.38)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.101_qs_sum_ovr_qol_oth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Getting on With Others Score		
Baseline		
n	9	9
Mean (SD)	81.9 (6.5)	78.9 (8.6)
Median	80.0	80.0
25th, 75th Percentile	78.3, 85.0	75.0, 86.7
Min, Max	72, 93	63, 88
Week 26		
n	11	12
Mean (SD)	76.7 (12.0)	77.9 (11.2)
Median	78.3	79.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.101_qs_sum_ovr_qol_oth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 83.3	69.2, 87.5
Min, Max	55, 97	60, 95
Change from baseline to Week 26 ^a		
n	7	9
Mean (SD)	-2.4 (8.6)	1.1 (6.3)
Median	-5.0	1.7
25th, 75th Percentile	-5.0, 1.7	0.0, 6.7
Min, Max	-15, 13	-10, 7
Week 52		
n	17	15
Mean (SD)	81.2 (9.6)	78.9 (11.1)
Median	80.0	80.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.101_qs_sum_ovr_qol_oth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 91.7	71.7, 88.3
Min, Max	63, 93	55, 92
Change from baseline to Week 52 ^a		
n	8	9
Mean (SD)	2.9 (10.9)	2.6 (7.0)
Median	4.2	1.7
25th, 75th Percentile	0.0, 10.0	0.0, 6.7
Min, Max	-20, 15	-7, 17
Difference in change from baseline (95%CI)		-0.32 (-9.69, 9.05)
P-value ^b		0.9426
Hedges'g (95% CI) ^c		-0.03 (-0.99, 0.92)
P-value for interaction term, treatment * [Sex]		0.3434

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.101_qs_sum_ovr_qol_oth_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Getting on With Others Score		
Baseline		
n	6	7
Mean (SD)	64.2 (19.5)	76.2 (6.5)
Median	70.0	73.3
25th, 75th Percentile	61.7, 75.0	70.0, 83.3
Min, Max	27, 82	70, 87
Week 26		
n	7	7
Mean (SD)	75.0 (11.9)	73.6 (6.5)
Median	73.3	73.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.102_qs_sum_ovr_qol_oth_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	68.3, 86.7	68.3, 80.0
Min, Max	55, 87	65, 83
Change from baseline to Week 26 ^a		
n	6	7
Mean (SD)	8.9 (14.2)	-2.6 (6.7)
Median	11.7	-5.0
25th, 75th Percentile	-6.7, 16.7	-6.7, 1.7
Min, Max	-8, 28	-10, 10
Week 52		
n	6	6
Mean (SD)	79.7 (10.8)	76.9 (11.1)
Median	77.5	75.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.102_qs_sum_ovr_qol_oth_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.0, 85.0	66.7, 83.3
Min, Max	70, 98	65, 95
Change from baseline to Week 52 ^a		
n	5	6
Mean (SD)	16.7 (21.5)	0.2 (12.9)
Median	8.3	-2.5
25th, 75th Percentile	3.3, 28.3	-10.0, 8.3
Min, Max	-5, 48	-13, 21
Difference in change from baseline (95%CI)		-16.45 (-40.09, 7.20)
P-value ^b		0.1500
Hedges'g (95% CI) ^c		-0.87 (-2.10, 0.40)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.102_qs_sum_ovr_qol_oth_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Getting on With Others Score		
Baseline		
n	8	8
Mean (SD)	82.1 (6.9)	79.4 (9.0)
Median	81.7	80.8
25th, 75th Percentile	77.5, 86.7	73.3, 87.5
Min, Max	72, 93	63, 88
Week 26		
n	7	8
Mean (SD)	77.9 (10.4)	80.0 (12.4)
Median	78.3	84.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.0, 83.3	69.2, 89.2
Min, Max	65, 97	60, 95
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	-5.0 (5.6)	0.6 (6.6)
Median	-5.0	1.7
25th, 75th Percentile	-5.0, -1.7	-4.2, 6.7
Min, Max	-15, 2	-10, 7
Week 52		
n	8	8
Mean (SD)	83.3 (11.3)	81.5 (7.9)
Median	85.8	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.002.102_qs_sum_ovr_qol_oth_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.2.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.8, 93.3	73.3, 87.5
Min, Max	63, 93	72, 92
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	1.4 (10.9)	2.1 (7.3)
Median	3.3	0.8
25th, 75th Percentile	-3.3, 6.7	-2.5, 5.0
Min, Max	-20, 15	-7, 17
Difference in change from baseline (95%CI)		0.66 (-9.56, 10.87)
P-value ^b		0.8917
Hedges'g (95% CI) ^c		0.07 (-0.95, 1.08)
P-value for interaction term, treatment *[Sex]		0.1150

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Getting on With Others Score		
Baseline		
n	14	11
Mean (SD)	75.4 (15.6)	76.5 (9.7)
Median	77.5	75.0
25th, 75th Percentile	71.7, 83.3	73.3, 86.7
Min, Max	27, 93	55, 88
Week 26		
n	16	15
Mean (SD)	77.9 (10.5)	76.3 (11.2)
Median	78.3	78.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.003.101_qs_sum_ovr_qol_oth_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.8, 85.0	66.7, 83.3
Min, Max	55, 97	55, 95
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	2.5 (11.1)	0.6 (5.6)
Median	-1.7	1.7
25th, 75th Percentile	-5.0, 9.2	-5.0, 5.0
Min, Max	-8, 28	-8, 10
Week 52		
n	22	19
Mean (SD)	80.2 (9.7)	78.6 (11.7)
Median	80.0	81.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.003.101_qs_sum_ovr_qol_oth_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 86.7	71.7, 88.3
Min, Max	62, 93	52, 95
Change from baseline to Week 52 ^a		
n	12	10
Mean (SD)	6.7 (15.8)	1.3 (8.2)
Median	3.3	0.0
25th, 75th Percentile	3.3, 10.0	-5.0, 3.3
Min, Max	-20, 48	-7, 21
Difference in change from baseline (95%CI)		-5.37 (-16.94, 6.20)
P-value ^b		0.3448
Hedges'g (95% CI) ^c		-0.40 (-1.24, 0.45)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.003.101_qs_sum_ovr_qol_oth_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Getting on With Others Score		
Baseline		
n	5	9
Mean (SD)	67.7 (18.2)	71.7 (11.4)
Median	70.0	70.0
25th, 75th Percentile	61.7, 80.0	65.0, 80.0
Min, Max	40, 87	50, 87
Week 26		
n	6	9
Mean (SD)	72.5 (11.5)	67.2 (13.1)
Median	74.2	70.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.003.101_qs_sum_ovr_qol_oth_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	65.0, 80.0	60.0, 73.3
Min, Max	55, 87	45, 87
Change from baseline to Week 26 ^a		
n	4	9
Mean (SD)	0.8 (14.3)	-4.4 (7.1)
Median	0.8	-6.7
25th, 75th Percentile	-10.8, 12.5	-10.0, -1.7
Min, Max	-15, 17	-13, 7
Week 52		
n	7	11
Mean (SD)	73.3 (18.7)	71.2 (11.2)
Median	76.7	73.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 81.7	63.3, 80.0
Min, Max	37, 98	52, 87
Change from baseline to Week 52 ^a		
n	5	9
Mean (SD)	5.0 (14.1)	2.2 (9.4)
Median	-3.3	1.7
25th, 75th Percentile	-3.3, 8.3	0.0, 8.3
Min, Max	-5, 28	-13, 17
Difference in change from baseline (95%CI)		-2.78 (-16.34, 10.78)
P-value ^b		0.6633
Hedges'g (95% CI) ^c		-0.23 (-1.33, 0.87)
P-value for interaction term, treatment * [Ethnicity]		0.7668

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Getting on With Others Score		
Baseline		
n	11	8
Mean (SD)	75.5 (17.6)	79.8 (7.4)
Median	78.3	79.2
25th, 75th Percentile	71.7, 85.0	73.3, 87.5
Min, Max	27, 93	70, 88
Week 26		
n	11	8
Mean (SD)	77.1 (11.2)	80.8 (10.6)
Median	78.3	82.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.003.102_qs_sum_ovr_qol_oth_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.0, 85.0	71.7, 89.2
Min, Max	55, 97	65, 95
Change from baseline to Week 26 ^a		
n	9	8
Mean (SD)	1.5 (12.3)	1.0 (5.8)
Median	-5.0	1.7
25th, 75th Percentile	-5.0, 1.7	-2.5, 4.2
Min, Max	-8, 28	-8, 10
Week 52		
n	11	7
Mean (SD)	81.8 (10.4)	81.9 (11.3)
Median	80.0	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 93.3	71.7, 91.7
Min, Max	63, 93	65, 95
Change from baseline to Week 52 ^a		
n	9	7
Mean (SD)	6.7 (18.3)	1.1 (9.6)
Median	3.3	0.0
25th, 75th Percentile	3.3, 6.7	-5.0, 3.3
Min, Max	-20, 48	-7, 21
Difference in change from baseline (95%CI)		-5.53 (-21.97, 10.92)
P-value ^b		0.4829
Hedges'g (95% CI) ^c		-0.34 (-1.33, 0.66)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Getting on With Others Score		
Baseline		
n	3	7
Mean (SD)	70.6 (9.2)	75.7 (8.3)
Median	70.0	76.7
25th, 75th Percentile	61.7, 80.0	70.0, 83.3
Min, Max	62, 80	63, 87
Week 26		
n	3	7
Mean (SD)	73.9 (11.3)	72.6 (8.6)
Median	70.0	70.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.3.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	65.0, 86.7	68.3, 80.0
Min, Max	65, 87	60, 87
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	3.3 (16.4)	-3.1 (7.2)
Median	8.3	-6.7
25th, 75th Percentile	-15.0, 16.7	-10.0, 6.7
Min, Max	-15, 17	-10, 7
Week 52		
n	3	7
Mean (SD)	81.7 (14.8)	77.1 (7.0)
Median	76.7	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.3.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.0, 98.3	71.7, 83.3
Min, Max	70, 98	67, 87
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	11.1 (16.0)	1.4 (10.5)
Median	8.3	1.7
25th, 75th Percentile	-3.3, 28.3	-10.0, 8.3
Min, Max	-3, 28	-13, 17
Difference in change from baseline (95%CI)		-9.68 (-28.93, 9.57)
P-value ^b		0.2796
Hedges'g (95% CI) ^c		-0.72 (-2.10, 0.69)
P-value for interaction term, treatment * [Ethnicity]		0.7345

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Getting on With Others Score		
Baseline		
n	3	8
Mean (SD)	54.4 (25.0)	77.5 (9.0)
Median	61.7	79.2
25th, 75th Percentile	26.7, 75.0	70.0, 85.0
Min, Max	27, 75	63, 88
Week 26		
n	4	8
Mean (SD)	72.5 (17.5)	74.2 (9.3)
Median	69.2	71.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.4.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.7, 83.3	69.2, 80.8
Min, Max	55, 97	60, 90
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	10.0 (17.6)	-3.3 (6.0)
Median	8.3	-4.2
25th, 75th Percentile	-6.7, 28.3	-8.3, 0.8
Min, Max	-7, 28	-10, 7
Week 52		
n	4	8
Mean (SD)	77.1 (11.1)	77.5 (7.7)
Median	72.5	76.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.4.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 84.2	72.5, 81.7
Min, Max	70, 93	67, 92
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	17.2 (27.8)	0.0 (9.9)
Median	8.3	0.8
25th, 75th Percentile	-5.0, 48.3	-8.3, 5.8
Min, Max	-5, 48	-13, 17
Difference in change from baseline (95%CI)		-17.22 (-82.15, 47.71)
P-value ^b		0.3955
Hedges'g (95% CI) ^c		-1.00 (-2.38, 0.43)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Getting on With Others Score		
Baseline		
n	11	7
Mean (SD)	79.8 (7.5)	78.3 (7.0)
Median	80.0	76.7
25th, 75th Percentile	71.7, 85.0	73.3, 86.7
Min, Max	70, 93	70, 88
Week 26		
n	10	7
Mean (SD)	78.0 (7.7)	80.2 (11.1)
Median	79.2	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.4.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	71.7, 85.0	68.3, 88.3
Min, Max	65, 87	65, 95
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	-0.7 (10.4)	1.9 (6.6)
Median	-5.0	1.7
25th, 75th Percentile	-5.0, 1.7	-5.0, 6.7
Min, Max	-15, 17	-8, 10
Week 52		
n	10	6
Mean (SD)	83.7 (10.7)	82.2 (11.3)
Median	82.5	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	76.7, 93.3	71.7, 88.3
Min, Max	63, 98	65, 95
Change from baseline to Week 52 ^a		
n	9	6
Mean (SD)	4.6 (13.0)	3.0 (9.9)
Median	3.3	0.0
25th, 75th Percentile	3.3, 6.7	-5.0, 6.7
Min, Max	-20, 28	-5, 21
Difference in change from baseline (95%CI)		-1.63 (-15.19, 11.93)
P-value ^b		0.7989
Hedges'g (95% CI) ^c		-0.13 (-1.16, 0.91)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.1952

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.004.101_qs_sum_ovr_qol_oth_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
ITQoL : Getting on With Others Score		
Baseline		
n	3	8
Mean (SD)	54.4 (25.0)	77.5 (9.0)
Median	61.7	79.2
25th, 75th Percentile	26.7, 75.0	70.0, 85.0
Min, Max	27, 75	63, 88
Week 26		
n	4	8
Mean (SD)	72.5 (17.5)	74.2 (9.3)
Median	69.2	71.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.4.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	61.7, 83.3	69.2, 80.8
Min, Max	55, 97	60, 90
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	10.0 (17.6)	-3.3 (6.0)
Median	8.3	-4.2
25th, 75th Percentile	-6.7, 28.3	-8.3, 0.8
Min, Max	-7, 28	-10, 7
Week 52		
n	4	8
Mean (SD)	77.1 (11.1)	77.5 (7.7)
Median	72.5	76.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	70.0, 84.2	72.5, 81.7
Min, Max	70, 93	67, 92
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	17.2 (27.8)	0.0 (9.9)
Median	8.3	0.8
25th, 75th Percentile	-5.0, 48.3	-8.3, 5.8
Min, Max	-5, 48	-13, 17
Difference in change from baseline (95%CI)		-17.22 (-82.15, 47.71)
P-value ^b		0.3955
Hedges'g (95% CI) ^c		-1.00 (-2.38, 0.43)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≥ 36 months to < 60 months		
ITQoL : Getting on With Others Score		
Baseline		
n	11	7
Mean (SD)	79.8 (7.5)	78.3 (7.0)
Median	80.0	76.7
25th, 75th Percentile	71.7, 85.0	73.3, 86.7
Min, Max	70, 93	70, 88
Week 26		
n	10	7
Mean (SD)	78.0 (7.7)	80.2 (11.1)
Median	79.2	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.4.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	71.7, 85.0	68.3, 88.3
Min, Max	65, 87	65, 95
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	-0.7 (10.4)	1.9 (6.6)
Median	-5.0	1.7
25th, 75th Percentile	-5.0, 1.7	-5.0, 6.7
Min, Max	-15, 17	-8, 10
Week 52		
n	10	6
Mean (SD)	83.7 (10.7)	82.2 (11.3)
Median	82.5	86.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.4.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	76.7, 93.3	71.7, 88.3
Min, Max	63, 98	65, 95
Change from baseline to Week 52 ^a		
n	9	6
Mean (SD)	4.6 (13.0)	3.0 (9.9)
Median	3.3	0.0
25th, 75th Percentile	3.3, 6.7	-5.0, 6.7
Min, Max	-20, 28	-5, 21
Difference in change from baseline (95%CI)		-1.63 (-15.19, 11.93)
P-value ^b		0.7989
Hedges'g (95% CI) ^c		-0.13 (-1.16, 0.91)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.1952

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Getting on With Others Score		
Baseline		
n	11	7
Mean (SD)	77.6 (6.3)	74.5 (12.4)
Median	76.7	76.7
25th, 75th Percentile	71.7, 83.3	70.0, 83.3
Min, Max	70, 88	50, 88
Week 26		
n	11	7
Mean (SD)	79.2 (8.4)	74.0 (16.5)
Median	78.3	73.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	71.7, 85.0	65.0, 86.7
Min, Max	68, 97	45, 95
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	0.7 (8.9)	-0.5 (8.0)
Median	-3.3	-5.0
25th, 75th Percentile	-5.0, 5.0	-6.7, 6.7
Min, Max	-8, 17	-10, 10
Week 52		
n	11	6
Mean (SD)	83.6 (11.4)	73.6 (14.7)
Median	85.0	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 93.3	65.0, 86.7
Min, Max	63, 98	52, 88
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	4.3 (12.4)	-1.1 (5.7)
Median	3.3	0.0
25th, 75th Percentile	3.3, 6.7	-5.0, 1.7
Min, Max	-20, 28	-10, 7
Difference in change from baseline (95%CI)		-5.44 (-17.07, 6.19)
P-value ^b		0.3325
Hedges'g (95% CI) ^c		-0.49 (-1.51, 0.55)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Getting on With Others Score		
Baseline		
n	8	13
Mean (SD)	67.5 (23.4)	74.2 (9.9)
Median	75.8	73.3
25th, 75th Percentile	50.8, 83.3	70.0, 81.7
Min, Max	27, 93	55, 88
Week 26		
n	11	17
Mean (SD)	73.6 (12.4)	72.5 (11.1)
Median	71.7	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	65.0, 85.0	66.7, 80.0
Min, Max	55, 93	52, 90
Change from baseline to Week 26 ^a		
n	6	13
Mean (SD)	4.4 (15.5)	-2.3 (6.1)
Median	3.3	0.0
25th, 75th Percentile	-6.7, 13.3	-6.7, 1.7
Min, Max	-15, 28	-13, 7
Week 52		
n	18	24
Mean (SD)	75.4 (12.3)	76.5 (11.4)
Median	76.7	76.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	71.7, 81.7	71.7, 84.5
Min, Max	37, 93	52, 95
Change from baseline to Week 52 ^a		
n	7	13
Mean (SD)	8.8 (18.7)	3.1 (9.4)
Median	3.3	1.7
25th, 75th Percentile	-3.3, 13.3	-3.3, 8.3
Min, Max	-5, 48	-13, 21
Difference in change from baseline (95%CI)		-5.76 (-23.26, 11.75)
P-value ^b		0.4677
Hedges'g (95% CI) ^c		-0.42 (-1.34, 0.52)
P-value for interaction term, treatment * [Baseline AGV Category]		0.9705

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≤ 4.5		
ITQoL : Getting on With Others Score		
Baseline		
n	10	6
Mean (SD)	78.0 (6.5)	78.6 (6.7)
Median	77.5	78.3
25th, 75th Percentile	71.7, 83.3	73.3, 83.3
Min, Max	70, 88	70, 88
Week 26		
n	10	6
Mean (SD)	79.3 (8.9)	78.9 (11.3)
Median	79.2	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.8.5.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	71.7, 85.0	70.0, 86.7
Min, Max	68, 97	65, 95
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	0.2 (9.3)	0.3 (8.5)
Median	-5.0	0.8
25th, 75th Percentile	-5.0, 1.7	-6.7, 6.7
Min, Max	-8, 17	-10, 10
Week 52		
n	10	5
Mean (SD)	84.3 (11.8)	78.0 (11.3)
Median	88.3	83.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.5.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	75.0, 93.3	66.7, 86.7
Min, Max	63, 98	65, 88
Change from baseline to Week 52 ^a		
n	9	5
Mean (SD)	4.4 (13.1)	-1.7 (6.2)
Median	3.3	0.0
25th, 75th Percentile	3.3, 6.7	-5.0, 0.0
Min, Max	-20, 28	-10, 7
Difference in change from baseline (95%CI)		-6.11 (-19.85, 7.63)
P-value ^b		0.3517
Hedges'g (95% CI) ^c		-0.51 (-1.61, 0.62)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.008.005.102_qs_sum_ovr_qol_oth_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.5.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Getting on With Others Score		
Baseline		
n	4	9
Mean (SD)	65.4 (28.9)	77.4 (8.9)
Median	70.8	76.7
25th, 75th Percentile	44.2, 86.7	70.0, 86.7
Min, Max	27, 93	63, 88
Week 26		
n	4	9
Mean (SD)	69.2 (13.2)	75.7 (10.0)
Median	67.5	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.5.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	60.0, 78.3	68.3, 81.7
Min, Max	55, 87	60, 90
Change from baseline to Week 26 ^a		
n	3	9
Mean (SD)	7.2 (21.7)	-1.7 (5.5)
Median	8.3	0.0
25th, 75th Percentile	-15.0, 28.3	-6.7, 1.7
Min, Max	-15, 28	-10, 7
Week 52		
n	4	9
Mean (SD)	75.4 (4.2)	80.3 (8.7)
Median	75.8	78.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.5.102
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	72.5, 78.3	73.3, 86.7
Min, Max	70, 80	72, 95
Change from baseline to Week 52 ^a		
n	3	9
Mean (SD)	17.8 (27.1)	2.9 (11.1)
Median	8.3	1.7
25th, 75th Percentile	-3.3, 48.3	-5.0, 8.3
Min, Max	-3, 48	-13, 21
Difference in change from baseline (95%CI)		-14.85 (-38.12, 8.42)
P-value ^b		0.1854
Hedges'g (95% CI) ^c		-0.88 (-2.22, 0.51)
P-value for interaction term, treatment * [Baseline AGV Category]		0.4628

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.6.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= -4		
ITQoL : Getting on With Others Score		
Baseline		
n	17	12
Mean (SD)	75.2 (14.8)	76.4 (8.6)
Median	78.3	75.0
25th, 75th Percentile	71.7, 83.3	70.0, 85.0
Min, Max	27, 93	63, 88
Week 26		
n	15	12
Mean (SD)	76.0 (9.6)	74.9 (12.5)
Median	78.3	74.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.6.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 83.3	67.5, 85.0
Min, Max	55, 93	52, 95
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	2.7 (11.6)	-1.5 (8.0)
Median	-1.7	-1.7
25th, 75th Percentile	-5.0, 13.3	-8.3, 6.7
Min, Max	-15, 28	-13, 10
Week 52		
n	15	12
Mean (SD)	81.9 (10.2)	79.5 (9.3)
Median	80.0	81.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.6.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	75.0, 93.3	72.5, 86.7
Min, Max	63, 98	65, 95
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	7.6 (15.4)	2.4 (10.4)
Median	3.3	0.0
25th, 75th Percentile	3.3, 13.3	-5.0, 8.3
Min, Max	-20, 48	-13, 21
Difference in change from baseline (95%CI)		-5.16 (-16.26, 5.94)
P-value ^b		0.3466
Hedges'g (95% CI) ^c		-0.37 (-1.15, 0.42)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.6.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Getting on With Others Score		
Baseline		
n	2	8
Mean (SD)	57.5 (24.7)	71.3 (12.9)
Median	57.5	74.2
25th, 75th Percentile	40.0, 75.0	62.5, 79.2
Min, Max	40, 75	50, 88
Week 26		
n	7	12
Mean (SD)	77.4 (13.7)	71.0 (12.8)
Median	78.3	71.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.6.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	68.3, 86.7	64.2, 80.8
Min, Max	55, 97	45, 90
Change from baseline to Week 26 ^a		
n	1	8
Mean (SD)	-6.7 (NA)	-1.9 (4.5)
Median	-6.7	-0.8
25th, 75th Percentile	-6.7, -6.7	-5.8, 0.8
Min, Max	-7, -7	-8, 5
Week 52		
n	14	18
Mean (SD)	74.9 (13.9)	73.5 (13.0)
Median	77.5	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.8.6.101
Infant Toddler Quality of Life (ITQoL): Getting Along with Others Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	70.0, 85.0	63.3, 81.7
Min, Max	37, 93	52, 92
Change from baseline to Week 52 ^a		
n	2	8
Mean (SD)	-4.2 (1.2)	0.8 (5.4)
Median	-4.2	1.7
25th, 75th Percentile	-5.0, -3.3	-4.2, 5.0
Min, Max	-5, -3	-7, 8
Difference in change from baseline (95%CI)		5.00 (-4.27, 14.27)
P-value ^b		0.2489
Hedges'g (95% CI) ^c		0.89 (-0.74, 2.47)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.3457

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Global Health Perceptions Score		
Baseline		
n	12	17
Mean (SD)	69.1 (18.1)	61.8 (14.2)
Median	67.8	63.6
25th, 75th Percentile	62.5, 77.3	50.0, 70.5
Min, Max	34, 98	39, 91
Week 26		
n	12	16
Mean (SD)	69.5 (12.9)	63.3 (12.2)
Median	64.8	67.8

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.4, 78.4	53.4, 70.5
Min, Max	52, 93	41, 80
Change from baseline to Week 26 ^a		
n	11	16
Mean (SD)	0.1 (11.3)	2.2 (14.4)
Median	-4.5	1.1
25th, 75th Percentile	-6.8, 11.4	-3.8, 10.2
Min, Max	-18, 18	-23, 34
Week 52		
n	13	15
Mean (SD)	71.7 (13.2)	66.1 (14.8)
Median	70.5	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.4, 75.0	56.8, 77.3
Min, Max	55, 100	36, 91
Change from baseline to Week 52 ^a		
n	12	15
Mean (SD)	2.9 (12.5)	3.0 (13.7)
Median	0.0	6.8
25th, 75th Percentile	-4.2, 9.1	-4.6, 11.4
Min, Max	-14, 27	-32, 25
Difference in change from baseline (95%CI)		0.15 (-10.35, 10.65)
P-value ^b		0.9769
Hedges'g (95% CI) ^c		0.01 (-0.75, 0.77)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Global Health Perceptions Score		
Baseline		
n	18	14
Mean (SD)	68.0 (15.7)	72.9 (13.8)
Median	68.2	72.7
25th, 75th Percentile	59.1, 79.6	65.9, 81.8
Min, Max	34, 91	45, 95
Week 26		
n	16	15
Mean (SD)	67.5 (15.2)	71.5 (12.8)
Median	69.3	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	54.6, 81.8	63.6, 77.3
Min, Max	41, 89	45, 95
Change from baseline to Week 26 ^a		
n	15	14
Mean (SD)	-2.1 (16.0)	-1.2 (13.6)
Median	-6.8	-1.1
25th, 75th Percentile	-11.4, 13.6	-6.8, 9.1
Min, Max	-30, 20	-30, 25
Week 52		
n	17	15
Mean (SD)	63.0 (14.4)	69.7 (15.6)
Median	65.9	72.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.002.101_qs_sum_ovr_qol_per_sex_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	52.3, 72.7	56.8, 81.8
Min, Max	34, 86	39, 95
Change from baseline to Week 52 ^a		
n	16	14
Mean (SD)	-5.7 (13.5)	-3.0 (12.1)
Median	-4.7	-4.5
25th, 75th Percentile	-11.4, 2.3	-9.1, 0.0
Min, Max	-32, 16	-25, 18
Difference in change from baseline (95%CI)		2.68 (-6.96, 12.32)
P-value ^b		0.5737
Hedges'g (95% CI) ^c		0.20 (-0.52, 0.92)
P-value for interaction term, treatment * [Sex]		0.7165

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Global Health Perceptions Score		
Baseline		
n	6	7
Mean (SD)	69.7 (25.8)	59.7 (14.0)
Median	70.5	61.4
25th, 75th Percentile	47.7, 97.7	47.7, 68.2
Min, Max	34, 98	39, 82
Week 26		
n	7	7
Mean (SD)	70.5 (15.5)	67.9 (11.4)
Median	65.9	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.2.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	61.4, 90.9	59.1, 79.6
Min, Max	52, 93	48, 80
Change from baseline to Week 26 ^a		
n	6	7
Mean (SD)	1.5 (11.3)	8.1 (14.5)
Median	-4.5	4.5
25th, 75th Percentile	-6.8, 13.6	-2.3, 18.2
Min, Max	-7, 18	-9, 34
Week 52		
n	7	6
Mean (SD)	75.0 (16.9)	70.5 (14.8)
Median	70.5	73.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	61.4, 95.5	56.8, 77.5
Min, Max	55, 100	50, 91
Change from baseline to Week 52 ^a		
n	6	6
Mean (SD)	6.4 (15.6)	7.2 (7.0)
Median	2.3	9.2
25th, 75th Percentile	-2.3, 22.7	9.1, 11.4
Min, Max	-14, 27	-7, 11
Difference in change from baseline (95%CI)		0.80 (-14.72, 16.32)
P-value ^b		0.9110
Hedges'g (95% CI) ^c		0.06 (-1.07, 1.19)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.2.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Global Health Perceptions Score		
Baseline		
n	8	8
Mean (SD)	61.7 (17.7)	71.7 (11.1)
Median	63.6	70.5
25th, 75th Percentile	47.7, 76.3	66.3, 79.5
Min, Max	34, 84	52, 89
Week 26		
n	7	8
Mean (SD)	62.7 (19.5)	71.9 (16.3)
Median	54.6	76.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.9.2.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	45.5, 84.1	60.2, 80.7
Min, Max	41, 89	45, 95
Change from baseline to Week 26 ^a		
n	6	8
Mean (SD)	-3.4 (16.5)	0.2 (13.4)
Median	-8.0	4.2
25th, 75th Percentile	-11.4, 11.1	-3.4, 9.1
Min, Max	-25, 20	-30, 11
Week 52		
n	8	8
Mean (SD)	57.1 (16.9)	71.6 (14.9)
Median	52.3	77.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.2.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	46.6, 69.3	56.8, 81.8
Min, Max	34, 86	50, 91
Change from baseline to Week 52 ^a		
n	7	8
Mean (SD)	-7.5 (13.0)	-0.1 (15.0)
Median	-4.8	-2.3
25th, 75th Percentile	-13.6, 2.3	-9.5, 14.8
Min, Max	-32, 9	-25, 18
Difference in change from baseline (95%CI)		7.41 (-8.41, 23.23)
P-value ^b		0.3302
Hedges'g (95% CI) ^c		0.49 (-0.55, 1.52)
P-value for interaction term, treatment * [Sex]		0.5274

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.3.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Global Health Perceptions Score		
Baseline		
n	23	20
Mean (SD)	71.4 (16.6)	68.4 (16.8)
Median	70.5	71.6
25th, 75th Percentile	63.6, 81.8	58.0, 78.4
Min, Max	34, 98	39, 95
Week 26		
n	22	20
Mean (SD)	71.0 (14.0)	68.2 (13.7)
Median	70.5	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.3.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.4, 84.1	63.3, 76.1
Min, Max	41, 93	41, 95
Change from baseline to Week 26 ^a		
n	20	19
Mean (SD)	-1.8 (14.7)	0.0 (14.6)
Median	-5.7	0.0
25th, 75th Percentile	-10.2, 11.3	-6.8, 9.1
Min, Max	-30, 20	-23, 34
Week 52		
n	23	19
Mean (SD)	67.4 (15.6)	68.2 (14.8)
Median	68.2	70.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.3.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	57.5, 77.3	56.8, 77.5
Min, Max	34, 100	39, 95
Change from baseline to Week 52 ^a		
n	21	18
Mean (SD)	-4.7 (13.0)	-1.9 (11.7)
Median	-3.9	-2.3
25th, 75th Percentile	-11.4, 2.3	-6.8, 4.5
Min, Max	-32, 23	-32, 18
Difference in change from baseline (95%CI)		2.79 (-5.30, 10.87)
P-value ^b		0.4894
Hedges'g (95% CI) ^c		0.22 (-0.41, 0.85)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.3.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Global Health Perceptions Score		
Baseline		
n	7	11
Mean (SD)	58.4 (11.9)	63.8 (10.8)
Median	61.4	63.6
25th, 75th Percentile	56.8, 63.6	56.8, 68.2
Min, Max	34, 73	50, 82
Week 26		
n	6	11
Mean (SD)	58.7 (9.9)	65.5 (12.0)
Median	58.0	65.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.3.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	52.3, 65.9	52.3, 77.3
Min, Max	45, 73	48, 80
Change from baseline to Week 26 ^a		
n	6	11
Mean (SD)	0.8 (12.2)	1.7 (13.3)
Median	-4.5	4.5
25th, 75th Percentile	-6.8, 13.6	-2.3, 9.1
Min, Max	-11, 18	-30, 18
Week 52		
n	7	11
Mean (SD)	64.6 (9.6)	67.4 (16.2)
Median	61.4	72.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.3.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	54.6, 75.0	56.8, 81.8
Min, Max	52, 75	36, 91
Change from baseline to Week 52 ^a		
n	7	11
Mean (SD)	6.2 (12.5)	3.5 (15.0)
Median	2.3	9.1
25th, 75th Percentile	-4.6, 15.9	-9.1, 13.6
Min, Max	-7, 27	-25, 25
Difference in change from baseline (95%CI)		-2.66 (-17.14, 11.82)
P-value ^b		0.7025
Hedges'g (95% CI) ^c		-0.18 (-1.13, 0.77)
P-value for interaction term, treatment * [Ethnicity]		0.4726

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.3.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Global Health Perceptions Score		
Baseline		
n	11	8
Mean (SD)	68.0 (21.3)	64.0 (16.6)
Median	70.5	67.4
25th, 75th Percentile	47.7, 84.1	50.0, 75.0
Min, Max	34, 98	39, 89
Week 26		
n	11	8
Mean (SD)	69.8 (17.8)	71.3 (15.2)
Median	65.9	70.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.3.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	54.6, 88.6	63.6, 80.7
Min, Max	41, 93	45, 95
Change from baseline to Week 26 ^a		
n	9	8
Mean (SD)	-1.3 (13.9)	7.3 (12.5)
Median	-4.6	4.2
25th, 75th Percentile	-6.8, 11.1	0.0, 11.4
Min, Max	-25, 20	-7, 34
Week 52		
n	12	7
Mean (SD)	66.1 (20.5)	69.5 (15.0)
Median	67.0	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.003.102_qs_sum_ovr_qol_per_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.3.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	51.1, 79.5	56.8, 79.6
Min, Max	34, 100	50, 91
Change from baseline to Week 52 ^a		
n	10	7
Mean (SD)	-3.9 (14.7)	1.9 (10.5)
Median	-3.5	-2.3
25th, 75th Percentile	-13.6, 2.3	-9.1, 9.3
Min, Max	-32, 23	-10, 18
Difference in change from baseline (95%CI)		5.76 (-8.12, 19.64)
P-value ^b		0.3902
Hedges'g (95% CI) ^c		0.41 (-0.57, 1.38)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.3.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Global Health Perceptions Score		
Baseline		
n	3	7
Mean (SD)	54.5 (19.4)	68.5 (9.8)
Median	56.8	65.9
25th, 75th Percentile	34.1, 72.7	61.4, 81.8
Min, Max	34, 73	57, 82
Week 26		
n	3	7
Mean (SD)	54.5 (10.4)	68.5 (13.3)
Median	52.3	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.3.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	45.5, 65.9	52.3, 79.6
Min, Max	45, 66	48, 80
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	0.0 (15.9)	0.0 (15.7)
Median	-6.8	4.5
25th, 75th Percentile	-11.4, 18.2	-9.1, 9.1
Min, Max	-11, 18	-30, 18
Week 52		
n	3	7
Mean (SD)	62.9 (11.4)	72.7 (14.6)
Median	61.4	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.3.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	52.3, 75.0	56.8, 81.8
Min, Max	52, 75	50, 91
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	8.3 (16.8)	4.2 (14.9)
Median	2.3	11.4
25th, 75th Percentile	-4.6, 27.3	-6.8, 13.6
Min, Max	-5, 27	-25, 16
Difference in change from baseline (95%CI)		-4.11 (-28.56, 20.34)
P-value ^b		0.7085
Hedges'g (95% CI) ^c		-0.24 (-1.59, 1.12)
P-value for interaction term, treatment * [Ethnicity]		0.4142

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Global Health Perceptions Score		
Baseline		
n	3	8
Mean (SD)	66.7 (31.8)	69.4 (9.6)
Median	68.2	66.3
25th, 75th Percentile	34.1, 97.7	62.5, 79.5
Min, Max	34, 98	57, 82
Week 26		
n	4	8
Mean (SD)	72.7 (17.9)	68.5 (12.3)
Median	73.9	71.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	58.0, 87.5	60.2, 78.4
Min, Max	52, 91	48, 80
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	2.3 (13.8)	-0.9 (14.1)
Median	-4.5	0.8
25th, 75th Percentile	-6.8, 18.2	-5.7, 6.8
Min, Max	-7, 18	-30, 18
Week 52		
n	4	8
Mean (SD)	75.6 (21.3)	69.9 (14.0)
Median	73.9	73.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	58.0, 93.2	56.8, 78.4
Min, Max	55, 100	50, 91
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	5.3 (20.6)	0.5 (14.0)
Median	2.3	3.4
25th, 75th Percentile	-13.6, 27.3	-8.3, 11.4
Min, Max	-14, 27	-25, 16
Difference in change from baseline (95%CI)		-4.83 (-28.86, 19.20)
P-value ^b		0.6602
Hedges'g (95% CI) ^c		-0.28 (-1.61, 1.06)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Global Health Perceptions Score		
Baseline		
n	11	7
Mean (SD)	64.7 (19.2)	62.3 (17.1)
Median	70.5	68.2
25th, 75th Percentile	47.7, 77.5	47.7, 72.7
Min, Max	34, 98	39, 89
Week 26		
n	10	7
Mean (SD)	64.1 (17.5)	71.8 (16.4)
Median	63.6	72.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.4.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	50.0, 75.0	59.1, 84.1
Min, Max	41, 93	45, 95
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	-2.0 (14.3)	9.4 (12.8)
Median	-6.8	9.1
25th, 75th Percentile	-9.1, 11.1	0.0, 11.4
Min, Max	-25, 20	-7, 34
Week 52		
n	11	6
Mean (SD)	61.8 (17.3)	72.8 (15.8)
Median	65.9	78.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.4.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	50.0, 72.7	56.8, 81.8
Min, Max	34, 95	50, 91
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-3.0 (14.3)	6.5 (10.2)
Median	-3.4	9.2
25th, 75th Percentile	-9.1, 2.3	-2.3, 13.6
Min, Max	-32, 23	-9, 18
Difference in change from baseline (95%CI)		9.46 (-4.96, 23.88)
P-value ^b		0.1812
Hedges'g (95% CI) ^c		0.69 (-0.37, 1.72)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.2469

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.4.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 24 months to < 36 months		
ITQoL : Global Health Perceptions Score		
Baseline		
n	3	8
Mean (SD)	66.7 (31.8)	69.4 (9.6)
Median	68.2	66.3
25th, 75th Percentile	34.1, 97.7	62.5, 79.5
Min, Max	34, 98	57, 82
Week 26		
n	4	8
Mean (SD)	72.7 (17.9)	68.5 (12.3)
Median	73.9	71.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	58.0, 87.5	60.2, 78.4
Min, Max	52, 91	48, 80
Change from baseline to Week 26 ^a		
n	3	8
Mean (SD)	2.3 (13.8)	-0.9 (14.1)
Median	-4.5	0.8
25th, 75th Percentile	-6.8, 18.2	-5.7, 6.8
Min, Max	-7, 18	-30, 18
Week 52		
n	4	8
Mean (SD)	75.6 (21.3)	69.9 (14.0)
Median	73.9	73.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.9.4.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	58.0, 93.2	56.8, 78.4
Min, Max	55, 100	50, 91
Change from baseline to Week 52 ^a		
n	3	8
Mean (SD)	5.3 (20.6)	0.5 (14.0)
Median	2.3	3.4
25th, 75th Percentile	-13.6, 27.3	-8.3, 11.4
Min, Max	-14, 27	-25, 16
Difference in change from baseline (95%CI)		-4.83 (-28.86, 19.20)
P-value ^b		0.6602
Hedges'g (95% CI) ^c		-0.28 (-1.61, 1.06)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Global Health Perceptions Score		
Baseline		
n	11	7
Mean (SD)	64.7 (19.2)	62.3 (17.1)
Median	70.5	68.2
25th, 75th Percentile	47.7, 77.5	47.7, 72.7
Min, Max	34, 98	39, 89
Week 26		
n	10	7
Mean (SD)	64.1 (17.5)	71.8 (16.4)
Median	63.6	72.7

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.4.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	50.0, 75.0	59.1, 84.1
Min, Max	41, 93	45, 95
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	-2.0 (14.3)	9.4 (12.8)
Median	-6.8	9.1
25th, 75th Percentile	-9.1, 11.1	0.0, 11.4
Min, Max	-25, 20	-7, 34
Week 52		
n	11	6
Mean (SD)	61.8 (17.3)	72.8 (15.8)
Median	65.9	78.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.004.102_qs_sum_ovr_qol_per_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.4.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	50.0, 72.7	56.8, 81.8
Min, Max	34, 95	50, 91
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	-3.0 (14.3)	6.5 (10.2)
Median	-3.4	9.2
25th, 75th Percentile	-9.1, 2.3	-2.3, 13.6
Min, Max	-32, 23	-9, 18
Difference in change from baseline (95%CI)		9.46 (-4.96, 23.88)
P-value ^b		0.1812
Hedges'g (95% CI) ^c		0.69 (-0.37, 1.72)
P-value for interaction term, treatment *[Cohort 1 Age Stratum]		0.2469

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.004.102_qs_sum_ovr_qol_per_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
<= 4.5		
ITQoL : Global Health Perceptions Score		
Baseline		
n	11	7
Mean (SD)	66.8 (19.6)	62.3 (18.4)
Median	72.7	61.4
25th, 75th Percentile	47.7, 81.8	47.7, 81.8
Min, Max	34, 98	39, 89
Week 26		
n	11	7
Mean (SD)	67.4 (16.4)	73.1 (15.5)
Median	63.6	77.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.005.101_qs_sum_ovr_qol_per_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.9.5.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	54.6, 84.1	59.1, 79.6
Min, Max	41, 93	48, 95
Change from baseline to Week 26 ^a		
n	10	7
Mean (SD)	-3.0 (14.2)	10.7 (12.6)
Median	-5.7	9.1
25th, 75th Percentile	-9.1, 11.1	-2.3, 18.2
Min, Max	-25, 20	-2, 34
Week 52		
n	12	6
Mean (SD)	64.2 (17.9)	69.7 (19.9)
Median	68.2	76.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.9.5.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	51.1, 73.9	56.8, 81.8
Min, Max	34, 95	36, 91
Change from baseline to Week 52 ^a		
n	11	6
Mean (SD)	-4.6 (14.2)	3.4 (11.7)
Median	-4.8	9.1
25th, 75th Percentile	-13.6, 2.3	-9.1, 11.4
Min, Max	-32, 23	-14, 14
Difference in change from baseline (95%CI)		7.98 (-6.49, 22.45)
P-value ^b		0.2582
Hedges'g (95% CI) ^c		0.57 (-0.46, 1.57)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.005.101_qs_sum_ovr_qol_per_agv_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> 4.5		
ITQoL : Global Health Perceptions Score		
Baseline		
n	19	24
Mean (SD)	69.4 (14.7)	68.1 (13.9)
Median	65.9	67.4
25th, 75th Percentile	61.4, 79.6	60.2, 75.0
Min, Max	34, 98	39, 95
Week 26		
n	17	24
Mean (SD)	69.0 (12.8)	65.6 (12.0)
Median	65.9	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.9.5.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.4, 79.6	61.4, 73.9
Min, Max	45, 91	41, 84
Change from baseline to Week 26 ^a		
n	16	23
Mean (SD)	-0.1 (14.2)	-2.5 (13.0)
Median	-4.5	-2.3
25th, 75th Percentile	-9.9, 12.5	-9.1, 6.8
Min, Max	-30, 20	-30, 25
Week 52		
n	18	24
Mean (SD)	68.5 (11.6)	67.4 (14.1)
Median	67.0	69.3

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.5.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.4, 75.0	56.8, 76.1
Min, Max	52, 100	39, 95
Change from baseline to Week 52 ^a		
n	17	23
Mean (SD)	-0.3 (13.3)	-0.7 (13.5)
Median	0.0	-2.3
25th, 75th Percentile	-4.6, 6.8	-6.8, 9.3
Min, Max	-30, 27	-32, 25
Difference in change from baseline (95%CI)		-0.39 (-9.08, 8.29)
P-value ^b		0.9274
Hedges'g (95% CI) ^c		-0.03 (-0.66, 0.60)
P-value for interaction term, treatment * [Baseline AGV Category]		0.3025

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
<= 4.5		
ITQoL : Global Health Perceptions Score		
Baseline		
n	10	6
Mean (SD)	65.3 (20.0)	64.4 (19.3)
Median	70.5	64.8
25th, 75th Percentile	47.7, 77.5	47.7, 81.8
Min, Max	34, 98	39, 89
Week 26		
n	10	6
Mean (SD)	67.7 (17.2)	77.3 (11.8)
Median	64.8	78.4

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	54.6, 84.1	72.7, 79.6
Min, Max	41, 93	59, 95
Change from baseline to Week 26 ^a		
n	9	6
Mean (SD)	-1.3 (13.9)	12.9 (12.3)
Median	-4.6	10.2
25th, 75th Percentile	-6.8, 11.1	6.8, 18.2
Min, Max	-25, 20	-2, 34
Week 52		
n	11	5
Mean (SD)	63.6 (18.7)	76.4 (12.7)
Median	65.9	79.6

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.005.102_qs_sum_ovr_qol_per_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	50.0, 75.0	72.7, 81.8
Min, Max	34, 95	57, 91
Change from baseline to Week 52 ^a		
n	10	5
Mean (SD)	-3.9 (14.7)	6.8 (9.1)
Median	-3.5	9.1
25th, 75th Percentile	-13.6, 2.3	9.1, 11.4
Min, Max	-32, 23	-9, 14
Difference in change from baseline (95%CI)		10.71 (-4.97, 26.39)
P-value ^b		0.1640
Hedges'g (95% CI) ^c		0.76 (-0.36, 1.86)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
> 4.5		
ITQoL : Global Health Perceptions Score		
Baseline		
n	4	9
Mean (SD)	64.8 (26.6)	67.3 (9.3)
Median	63.6	66.7
25th, 75th Percentile	45.5, 84.1	63.6, 72.7
Min, Max	34, 98	52, 82
Week 26		
n	4	9
Mean (SD)	63.6 (20.1)	65.2 (13.6)
Median	59.1	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.005.102_qs_sum_ovr_qol_per_agv_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.9.5.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	48.9, 78.4	52.3, 75.0
Min, Max	45, 91	45, 84
Change from baseline to Week 26 ^a		
n	3	9
Mean (SD)	0.0 (15.9)	-2.1 (12.2)
Median	-6.8	0.0
25th, 75th Percentile	-11.4, 18.2	-6.8, 4.5
Min, Max	-11, 18	-30, 11
Week 52		
n	4	9
Mean (SD)	70.5 (20.7)	68.2 (15.0)
Median	64.8	75.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.5.102
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline AGV Category Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	56.8, 84.1	56.8, 77.5
Min, Max	52, 100	50, 91
Change from baseline to Week 52 ^a		
n	3	9
Mean (SD)	8.3 (16.8)	1.0 (14.0)
Median	2.3	-2.3
25th, 75th Percentile	-4.6, 27.3	-6.8, 11.4
Min, Max	-5, 27	-25, 18
Difference in change from baseline (95%CI)		-7.38 (-29.07, 14.31)
P-value ^b		0.4659
Hedges'g (95% CI) ^c		-0.47 (-1.78, 0.87)
P-value for interaction term, treatment * [Baseline AGV Category]		0.1439

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.6.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category	Placebo (N=32)	Vosoritide (N=32)
Score		
Visit		
Result		
<hr/>		
<= -4		
ITQoL : Global Health Perceptions Score		
Baseline		
n	18	13
Mean (SD)	66.5 (19.0)	66.3 (14.1)
Median	69.0	68.2
25th, 75th Percentile	47.7, 77.5	56.8, 72.7
Min, Max	34, 98	39, 89
Week 26		
n	15	12
Mean (SD)	65.8 (16.9)	71.2 (13.7)
Median	61.4	73.9

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.6.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	52.3, 84.1	61.4, 79.6
Min, Max	41, 93	48, 95
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	-1.0 (14.3)	5.5 (15.4)
Median	-6.8	8.0
25th, 75th Percentile	-9.1, 13.6	-1.1, 11.4
Min, Max	-25, 20	-30, 34
Week 52		
n	16	12
Mean (SD)	64.6 (17.1)	72.6 (13.6)
Median	64.8	75.1

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.009.006.101_qs_sum_ovr_qol_per_haz_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.6.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	52.3, 71.6	60.2, 81.8
Min, Max	34, 100	50, 91
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	-1.0 (13.7)	4.0 (12.7)
Median	-2.3	9.1
25th, 75th Percentile	-6.9, 2.3	-5.7, 12.5
Min, Max	-32, 27	-25, 18
Difference in change from baseline (95%CI)		4.97 (-5.48, 15.42)
P-value ^b		0.3377
Hedges'g (95% CI) ^c		0.36 (-0.40, 1.11)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.9.6.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
> -4		
ITQoL : Global Health Perceptions Score		
Baseline		
n	12	18
Mean (SD)	71.2 (11.7)	67.2 (15.9)
Median	67.0	66.3
25th, 75th Percentile	61.4, 80.7	52.3, 77.3
Min, Max	59, 91	39, 95
Week 26		
n	13	19
Mean (SD)	71.3 (9.5)	64.8 (12.2)
Median	72.7	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.6.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	63.6, 79.6	59.1, 72.7
Min, Max	55, 84	41, 84
Change from baseline to Week 26 ^a		
n	11	18
Mean (SD)	-1.4 (14.2)	-2.6 (12.2)
Median	-4.5	-2.3
25th, 75th Percentile	-11.4, 11.4	-6.8, 2.3
Min, Max	-30, 18	-23, 25
Week 52		
n	14	18
Mean (SD)	69.2 (10.4)	64.8 (15.6)
Median	68.2	68.2

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.9.6.101
Infant Toddler Quality of Life (ITQoL): Global Health Perception Score (Overall) by Baseline Height Z-Score Category for BMN111-206
Analysis Population: Full Analysis Set

Baseline Height Z-Score Category Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	61.4, 77.3	56.8, 75.0
Min, Max	55, 86	36, 95
Change from baseline to Week 52 ^a		
n	12	17
Mean (SD)	-3.4 (13.7)	-2.6 (13.0)
Median	-2.3	-2.3
25th, 75th Percentile	-11.4, 8.0	-9.1, 4.5
Min, Max	-30, 16	-32, 25
Difference in change from baseline (95%CI)		0.77 (-9.52, 11.05)
P-value ^b		0.8796
Hedges'g (95% CI) ^c		0.06 (-0.68, 0.79)
P-value for interaction term, treatment * [Baseline Height Z-Score Category]		0.5589

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Male		
ITQoL : Change in Health Score		
Baseline		
n	10	11
Mean (SD)	3.7 (0.9)	4.0 (0.8)
Median	3.0	4.0
25th, 75th Percentile	3.0, 5.0	3.0, 5.0
Min, Max	3, 5	3, 5
Week 26		
n	11	12
Mean (SD)	3.6 (0.7)	4.0 (0.6)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.10.2.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	4.0, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 26 ^a		
n	9	10
Mean (SD)	0.2 (1.0)	0.0 (0.9)
Median	0.0	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 1.0
Min, Max	-2, 1	-2, 1
Week 52		
n	13	15
Mean (SD)	3.5 (0.7)	3.7 (0.8)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.10.2.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	10	10
Mean (SD)	-0.2 (1.0)	0.0 (0.7)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-2, 1	-1, 1
Difference in change from baseline (95%CI)		0.20 (-0.62, 1.02)
P-value ^b		0.6132
Hedges'g (95% CI) ^c		0.22 (-0.66, 1.10)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Female		
ITQoL : Change in Health Score		
Baseline		
n	10	8
Mean (SD)	3.9 (1.0)	3.4 (0.5)
Median	3.5	3.0
25th, 75th Percentile	3.0, 5.0	3.0, 4.0
Min, Max	3, 5	3, 4
Week 26		
n	8	12
Mean (SD)	3.9 (1.0)	3.3 (0.5)
Median	3.5	3.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Table 14.2.13.10.2.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 5.0	3.0, 3.5
Min, Max	3, 5	3, 4
Change from baseline to Week 26 ^a		
n	7	7
Mean (SD)	0.3 (1.0)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	-1, 2	-1, 1
Week 52		
n	16	15
Mean (SD)	3.6 (0.7)	3.5 (0.8)
Median	3.5	3.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	8	8
Mean (SD)	0.0 (0.8)	0.4 (0.5)
Median	0.0	0.0
25th, 75th Percentile	-0.5, 0.5	0.0, 1.0
Min, Max	-1, 1	0, 1
Difference in change from baseline (95%CI)		0.38 (-0.32, 1.07)
P-value ^b		0.2663
Hedges'g (95% CI) ^c		0.55 (-0.46, 1.54)
P-value for interaction term, treatment * [Sex]		0.7403

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Male		
ITQoL : Change in Health Score		
Baseline		
n	6	7
Mean (SD)	3.2 (0.4)	4.3 (0.5)
Median	3.0	4.0
25th, 75th Percentile	3.0, 3.0	4.0, 5.0
Min, Max	3, 4	4, 5
Week 26		
n	7	7
Mean (SD)	3.6 (0.5)	4.3 (0.5)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	4.0, 5.0
Min, Max	3, 4	4, 5
Change from baseline to Week 26 ^a		
n	6	7
Mean (SD)	0.5 (0.5)	0.0 (0.6)
Median	0.5	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	0, 1	-1, 1
Week 52		
n	7	6
Mean (SD)	3.4 (0.5)	4.0 (0.6)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.13.10.2.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	4.0, 4.0
Min, Max	3, 4	3, 5
Change from baseline to Week 52 ^a		
n	6	6
Mean (SD)	0.3 (0.5)	-0.2 (0.8)
Median	0.0	0.0
25th, 75th Percentile	0.0, 1.0	-1.0, 0.0
Min, Max	0, 1	-1, 1
Difference in change from baseline (95%CI)		-0.50 (-1.33, 0.33)
P-value ^b		0.2094
Hedges'g (95% CI) ^c		-0.72 (-1.87, 0.47)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Female		
ITQoL : Change in Health Score		
Baseline		
n	8	7
Mean (SD)	3.8 (1.0)	3.4 (0.5)
Median	3.0	3.0
25th, 75th Percentile	3.0, 5.0	3.0, 4.0
Min, Max	3, 5	3, 4
Week 26		
n	6	8
Mean (SD)	3.8 (1.0)	3.4 (0.5)
Median	3.5	3.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 5.0	3.0, 4.0
Min, Max	3, 5	3, 4
Change from baseline to Week 26 ^a		
n	6	7
Mean (SD)	0.2 (1.0)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-1, 2	-1, 1
Week 52		
n	8	8
Mean (SD)	3.6 (0.7)	3.8 (0.9)
Median	3.5	3.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.002.102_qs_sum_ovr_qol_chg_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.2.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (≥ 24 to < 60 months)) by Sex for BMN111-206
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.0, 4.5
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	7	7
Mean (SD)	-0.1 (0.7)	0.4 (0.5)
Median	0.0	0.0
25th, 75th Percentile	-1.0, 0.0	0.0, 1.0
Min, Max	-1, 1	0, 1
Difference in change from baseline (95%CI)		0.57 (-0.15, 1.29)
P-value ^b		0.1089
Hedges'g (95% CI) ^c		0.87 (-0.25, 1.95)
P-value for interaction term, treatment * [Sex]		0.0419

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.002.102_qs_sum_ovr_qol_chg_sex_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
White		
ITQoL : Change in Health Score		
Baseline		
n	14	11
Mean (SD)	3.6 (0.9)	3.6 (0.8)
Median	3.0	3.0
25th, 75th Percentile	3.0, 5.0	3.0, 4.0
Min, Max	3, 5	3, 5
Week 26		
n	15	14
Mean (SD)	3.9 (0.8)	3.6 (0.6)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.101_qs_sum_ovr_qol_chg_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 5.0	3.0, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 26 ^a		
n	12	9
Mean (SD)	0.5 (0.8)	0.1 (0.6)
Median	0.5	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	-1, 2	-1, 1
Week 52		
n	23	19
Mean (SD)	3.6 (0.7)	3.7 (0.9)
Median	3.0	3.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.101_qs_sum_ovr_qol_chg_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	3.0, 5.0
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	0.1 (0.6)	0.4 (0.7)
Median	0.0	0.5
25th, 75th Percentile	0.0, 0.0	0.0, 1.0
Min, Max	-1, 1	-1, 1
Difference in change from baseline (95%CI)		0.32 (-0.26, 0.91)
P-value ^b		0.2620
Hedges'g (95% CI) ^c		0.47 (-0.37, 1.30)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.101_qs_sum_ovr_qol_chg_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
Non-White		
ITQoL : Change in Health Score		
Baseline		
n	6	8
Mean (SD)	4.2 (1.0)	3.9 (0.6)
Median	4.5	4.0
25th, 75th Percentile	3.0, 5.0	3.5, 4.0
Min, Max	3, 5	3, 5
Week 26		
n	4	10
Mean (SD)	3.3 (0.5)	3.6 (0.7)
Median	3.0	3.5

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.101_qs_sum_ovr_qol_chg_eth_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 3.5	3.0, 4.0
Min, Max	3, 4	3, 5
Change from baseline to Week 26 ^a		
n	4	8
Mean (SD)	-0.5 (1.0)	-0.1 (1.0)
Median	0.0	0.0
25th, 75th Percentile	-1.0, 0.0	-0.5, 0.5
Min, Max	-2, 0	-2, 1
Week 52		
n	6	11
Mean (SD)	3.5 (0.5)	3.5 (0.7)
Median	3.5	3.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 4	3, 5
Change from baseline to Week 52 ^a		
n	5	8
Mean (SD)	-0.6 (1.3)	-0.1 (0.4)
Median	0.0	0.0
25th, 75th Percentile	-2.0, 0.0	0.0, 0.0
Min, Max	-2, 1	-1, 0
Difference in change from baseline (95%CI)		0.48 (-1.17, 2.12)
P-value ^b		0.4783
Hedges'g (95% CI) ^c		0.52 (-0.63, 1.64)
P-value for interaction term, treatment * [Ethnicity]		0.7731

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
White		
ITQoL : Change in Health Score		
Baseline		
n	11	8
Mean (SD)	3.5 (0.9)	3.9 (0.8)
Median	3.0	4.0
25th, 75th Percentile	3.0, 5.0	3.0, 4.5
Min, Max	3, 5	3, 5
Week 26		
n	10	8
Mean (SD)	3.8 (0.8)	3.9 (0.6)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.102_qs_sum_ovr_qol_chg_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.5, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 26 ^a		
n	9	8
Mean (SD)	0.4 (0.9)	0.0 (0.5)
Median	0.0	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	-1, 2	-1, 1
Week 52		
n	12	7
Mean (SD)	3.5 (0.7)	4.1 (0.9)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.0, 5.0
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	10	7
Mean (SD)	0.0 (0.7)	0.4 (0.8)
Median	0.0	1.0
25th, 75th Percentile	0.0, 0.0	0.0, 1.0
Min, Max	-1, 1	-1, 1
Difference in change from baseline (95%CI)		0.43 (-0.32, 1.18)
P-value ^b		0.2440
Hedges'g (95% CI) ^c		0.57 (-0.43, 1.54)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.102_qs_sum_ovr_qol_chg_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
Non-White		
ITQoL : Change in Health Score		
Baseline		
n	3	6
Mean (SD)	3.3 (0.6)	3.8 (0.4)
Median	3.0	4.0
25th, 75th Percentile	3.0, 4.0	4.0, 4.0
Min, Max	3, 4	3, 4
Week 26		
n	3	7
Mean (SD)	3.3 (0.6)	3.7 (0.8)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.102_qs_sum_ovr_qol_chg_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 4	3, 5
Change from baseline to Week 26 ^a		
n	3	6
Mean (SD)	0.0 (0.0)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	0, 0	-1, 1
Week 52		
n	3	7
Mean (SD)	3.7 (0.6)	3.6 (0.5)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.102_qs_sum_ovr_qol_chg_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.3.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Ethnicity for BMN111-206
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 4	3, 4
Change from baseline to Week 52 ^a		
n	3	6
Mean (SD)	0.3 (0.6)	-0.2 (0.4)
Median	0.0	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	0, 1	-1, 0
Difference in change from baseline (95%CI)		-0.50 (-1.27, 0.27)
P-value ^b		0.1705
Hedges'g (95% CI) ^c		-0.96 (-2.40, 0.54)
P-value for interaction term, treatment * [Ethnicity]		0.1101

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.003.102_qs_sum_ovr_qol_chg_eth_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 24 months to < 36 months		
ITQoL : Change in Health Score		
Baseline		
n	3	7
Mean (SD)	3.3 (0.6)	3.9 (0.4)
Median	3.0	4.0
25th, 75th Percentile	3.0, 4.0	4.0, 4.0
Min, Max	3, 4	3, 4
Week 26		
n	3	8
Mean (SD)	4.0 (0.0)	3.8 (0.7)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.101_qs_sum_ovr_qol_chg_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.10.4.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	4.0, 4.0	3.0, 4.0
Min, Max	4, 4	3, 5
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	0.7 (0.6)	0.0 (0.6)
Median	1.0	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	0, 1	-1, 1
Week 52		
n	4	8
Mean (SD)	3.3 (0.5)	3.8 (0.7)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.101_qs_sum_ovr_qol_chg_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 3.5	3.0, 4.0
Min, Max	3, 4	3, 5
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	0.0 (0.0)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	0, 0	-1, 1
Difference in change from baseline (95%CI)		0.00 (-0.53, 0.53)
P-value ^b		1.0000
Hedges'g (95% CI) ^c		0.00 (-1.35, 1.35)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.101_qs_sum_ovr_qol_chg_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
>= 36 months to < 60 months		
ITQoL : Change in Health Score		
Baseline		
n	11	7
Mean (SD)	3.5 (0.9)	3.9 (0.9)
Median	3.0	4.0
25th, 75th Percentile	3.0, 5.0	3.0, 5.0
Min, Max	3, 5	3, 5
Week 26		
n	10	7
Mean (SD)	3.6 (0.8)	3.9 (0.7)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.101_qs_sum_ovr_qol_chg_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.10.4.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	0.2 (0.8)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-1, 2	-1, 1
Week 52		
n	11	6
Mean (SD)	3.6 (0.7)	4.0 (0.9)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.101_qs_sum_ovr_qol_chg_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.101
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Overall) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=32)	Vosoritide (N=32)
25th, 75th Percentile	3.0, 4.0	3.0, 5.0
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	0.1 (0.7)	0.3 (0.8)
Median	0.0	0.5
25th, 75th Percentile	0.0, 1.0	0.0, 1.0
Min, Max	-1, 1	-1, 1
Difference in change from baseline (95%CI)		0.23 (-0.62, 1.08)
P-value ^b		0.5651
Hedges'g (95% CI) ^c		0.29 (-0.73, 1.30)
P-value for interaction term, treatment * [Cohort 1 Age Stratum]		0.6952

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.101_qs_sum_ovr_qol_chg_strat_ov_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
≥ 24 months to < 36 months		
ITQoL : Change in Health Score		
Baseline		
n	3	7
Mean (SD)	3.3 (0.6)	3.9 (0.4)
Median	3.0	4.0
25th, 75th Percentile	3.0, 4.0	4.0, 4.0
Min, Max	3, 4	3, 4
Week 26		
n	3	8
Mean (SD)	4.0 (0.0)	3.8 (0.7)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.102_qs_sum_ovr_qol_chg_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.10.4.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	4.0, 4.0	3.0, 4.0
Min, Max	4, 4	3, 5
Change from baseline to Week 26 ^a		
n	3	7
Mean (SD)	0.7 (0.6)	0.0 (0.6)
Median	1.0	0.0
25th, 75th Percentile	0.0, 1.0	0.0, 0.0
Min, Max	0, 1	-1, 1
Week 52		
n	4	8
Mean (SD)	3.3 (0.5)	3.8 (0.7)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.102_qs_sum_ovr_qol_chg_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 3.5	3.0, 4.0
Min, Max	3, 4	3, 5
Change from baseline to Week 52 ^a		
n	3	7
Mean (SD)	0.0 (0.0)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	0, 0	-1, 1
Difference in change from baseline (95%CI)		0.00 (-0.53, 0.53)
P-value ^b		1.0000
Hedges'g (95% CI) ^c		0.00 (-1.35, 1.35)

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.102_qs_sum_ovr_qol_chg_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
>= 36 months to < 60 months		
ITQoL : Change in Health Score		
Baseline		
n	11	7
Mean (SD)	3.5 (0.9)	3.9 (0.9)
Median	3.0	4.0
25th, 75th Percentile	3.0, 5.0	3.0, 5.0
Min, Max	3, 5	3, 5
Week 26		
n	10	7
Mean (SD)	3.6 (0.8)	3.9 (0.7)
Median	3.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.102_qs_sum_ovr_qol_chg_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

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Table 14.2.13.10.4.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.0, 4.0
Min, Max	3, 5	3, 5
Change from baseline to Week 26 ^a		
n	9	7
Mean (SD)	0.2 (0.8)	0.0 (0.6)
Median	0.0	0.0
25th, 75th Percentile	0.0, 0.0	0.0, 0.0
Min, Max	-1, 2	-1, 1
Week 52		
n	11	6
Mean (SD)	3.6 (0.7)	4.0 (0.9)
Median	4.0	4.0

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.102_qs_sum_ovr_qol_chg_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.2.13.10.4.102
Infant Toddler Quality of Life (ITQoL): Change in Health Score (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum for BMN111-206
Analysis Population: Full Analysis Set

Cohort 1 Age Stratum Score Visit Result	Placebo (N=16)	Vosoritide (N=15)
25th, 75th Percentile	3.0, 4.0	3.0, 5.0
Min, Max	3, 5	3, 5
Change from baseline to Week 52 ^a		
n	10	6
Mean (SD)	0.1 (0.7)	0.3 (0.8)
Median	0.0	0.5
25th, 75th Percentile	0.0, 1.0	0.0, 1.0
Min, Max	-1, 1	-1, 1
Difference in change from baseline (95%CI)		0.23 (-0.62, 1.08)
P-value ^b		0.5651
Hedges'g (95% CI) ^c		0.29 (-0.73, 1.30)
P-value for interaction term, treatment *[Cohort 1 Age Stratum]		0.6952

Max, maximum; Min, minimum; SD, standard deviation; NE, not estimable.

A higher score reflects a higher quality of life.

^a Change from baseline was based on the subjects with available measurements at both time points. Baseline is defined as Day 1 or screening if a Day 1 assessment is not available.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

The p-value for the interaction term is based from an analysis of variance model, which only includes the terms used for interaction as covariates.

Report: mi897809 21JUN2023 11:46 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.13.010.004.102_qs_sum_ovr_qol_chg_strat_c1_206_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_206.sas, Database: N/A

Table 14.3.2.101.6

Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Event of Interest (Overall): Injection site reactions for BMN111-206
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3040
Race*Treatment Interaction	0.1958
Cohort 1 age stratum *Treatment Interaction	0.6080
Baseline AGV Category*Treatment Interaction	0.1591
Baseline Height Z-Score Category*Treatment Interaction	0.2940

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.006.000_ae_eoiisr_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.101.7

Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Event of Interest (Overall): Hypersensitivity (SMQ Narrow Terms) for
BMN111-206

Analysis Population: Safety Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5069
Race*Treatment Interaction	0.0394
Cohort 1 age stratum *Treatment Interaction	0.1304
Baseline AGV Category*Treatment Interaction	0.1962
Baseline Height Z-Score Category*Treatment Interaction	0.7061

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.007.000_ae_eoihyp_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.102.1

Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing Any Adverse Event (Cohort 1 (≥ 24 to < 60 months)) for BMN111-206
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	NA
Race*Treatment Interaction	NA
Cohort 1 age stratum *Treatment Interaction	NA
Baseline AGV Category*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	NA

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.102.001.000_ae_aae_c1_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.102.2
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event (Cohort 1 (≥ 24 to < 60 months)): Injection Site
Reaction for BMN111-206
Analysis Population: Safety Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.4680
Race*Treatment Interaction	NE
Cohort 1 age stratum *Treatment Interaction	0.4160
Baseline AGV Category*Treatment Interaction	0.2346
Baseline Height Z-Score Category*Treatment Interaction	0.3569

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.102.002.000_ae_isr_c1_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.102.3
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event (Cohort 1 (≥ 24 to < 60 months)): Injection site erythema for BMN111-206
Analysis Population: Safety Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.4244
Race*Treatment Interaction	NE
Cohort 1 age stratum *Treatment Interaction	0.6455
Baseline AGV Category*Treatment Interaction	0.4748
Baseline Height Z-Score Category*Treatment Interaction	0.0838

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.102.003.000_ae_ise_c1_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.102.4

Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Event of Interest (Cohort 1 (≥ 24 to < 60 months)): Injection site reactions for BMN111-206

Analysis Population: Safety Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.8296
Race*Treatment Interaction	NE
Cohort 1 age stratum *Treatment Interaction	0.6080
Baseline AGV Category*Treatment Interaction	0.4049

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.102.004.000_ae_eoiisr_c1_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.1.1.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Sex: Male for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=13)	Vosoritide (N=25)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	13 (100.0)	25 (100.0)	NA	NA	NA
Injection site erythema	5 (38.5)	20 (80.0)	2.08 (1.02; 4.25) 0.0447	6.40 (1.45; 28.29) 0.0144	0.4154 (0.11; 0.72) 0.0081
Injection site reaction	5 (38.5)	20 (80.0)	2.08 (1.02; 4.25) 0.0447	6.40 (1.45; 28.29) 0.0144	0.4154 (0.11; 0.72) 0.0081
Gastrointestinal disorders, n (%) ^a	11 (84.6)	15 (60.0)	0.71 (0.48; 1.05)	0.27 (0.05; 1.50)	-0.2462 (-0.52; 0.03)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.001.701.001_ae_soc_pt_male_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.1.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Sex: Male for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=13)	Vosoritide (N=25)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.0882	0.1355	0.0788
Vomiting	7 (53.8)	7 (28.0)	0.52 (0.23; 1.16)	0.33 (0.08; 1.35)	-0.2585 (-0.58; 0.06)
			0.1115	0.1232	0.1170

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.001.701.001_ae_soc_pt_male_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.1.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Sex: Female for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=19)	Vosoritide (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	19 (100.0)	18 (100.0)	NA	NA	NA
Injection site reaction	8 (42.1)	14 (77.8)	1.85 (1.03; 3.31) 0.0388	4.81 (1.14; 20.25) 0.0321	0.3567 (0.06; 0.65) 0.0172
Injection site erythema	8 (42.1)	13 (72.2)	1.72 (0.94; 3.13) 0.0780	3.58 (0.90; 14.15) 0.0696	0.3012 (0.00; 0.60) 0.0518
Gastrointestinal disorders, n (%) ^a	15 (78.9)	10 (55.6)	0.70 (0.44; 1.13)	0.33 (0.08; 1.41)	-0.2339 (-0.53; 0.06)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.001.701.002_ae_soc_pt_female_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.1.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Sex: Female for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=19)	Vosoritide (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.1462	0.1355	0.1186
Vomiting	10 (52.6)	4 (22.2)	0.42 (0.16; 1.11)	0.26 (0.06; 1.07)	-0.3041 (-0.60; -0.01)
			0.0795	0.0627	0.0437

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.001.701.002_ae_soc_pt_female_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.1.702.1
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Sex: Male for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=7)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	7 (100.0)	10 (100.0)	NA	NA	NA
Injection site reaction	4 (57.1)	10 (100.0)	1.75 (1.04; 7.62)	NA	0.4286 (0.03; 0.82)
Injection site erythema	3 (42.9)	9 (90.0)	2.10 (0.87; 5.06)	12.00 (0.94; 153.9)	0.4714 (0.06; 0.88)
			0.0984	0.0563	0.0246

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.1.702.2
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Sex: Female for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=9)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	9 (100.0)	9 (100.0)	NA	NA	NA
Injection site reaction	2 (22.2)	6 (66.7)	3.00 (0.81; 11.08) 0.0994	7.00 (0.86; 56.89) 0.0687	0.4444 (0.03; 0.86) 0.0339
Injection site erythema	1 (11.1)	5 (55.6)	5.00 (0.72; 34.73) 0.1036	10.00 (0.85; 117.0) 0.0665	0.4444 (0.06; 0.83) 0.0233

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.001.702.002_ae_soc_pt_female_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.2.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Ethnicity: White for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=25)	Vosoritide (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	25 (100.0)	29 (100.0)	NA	NA	NA
Injection site reaction	12 (48.0)	27 (93.1)	1.94 (1.27; 2.95) 0.0020	14.63 (2.85; 75.14) 0.0013	0.4510 (0.23; 0.67) 0.0000
Injection site erythema	12 (48.0)	25 (86.2)	1.80 (1.16; 2.77) 0.0081	6.77 (1.82; 25.22) 0.0044	0.3821 (0.15; 0.61) 0.0013
Gastrointestinal disorders, n (%) ^a	20 (80.0)	19 (65.5)	0.82 (0.59; 1.14)	0.48 (0.14; 1.65)	-0.1448 (-0.38; 0.09)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.2.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Ethnicity: White for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=25)	Vosoritide (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.2339	0.2407	0.2241
Vomiting	13 (52.0)	10 (34.5)	0.66 (0.35; 1.24)	0.49 (0.16; 1.45)	-0.1752 (-0.44; 0.09)
			0.1993	0.1969	0.1889

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.2.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Ethnicity: Non-White for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=7)	Vosoritide (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	7 (100.0)	14 (100.0)	NA	NA	NA
Injection site erythema	1 (14.3)	8 (57.1)	4.00 (0.62; 25.96) 0.1463	8.00 (0.75; 85.31) 0.0851	0.4286 (0.06; 0.80) 0.0219
Injection site reaction	1 (14.3)	7 (50.0)	3.50 (0.53; 23.14) 0.1936	6.00 (0.57; 63.68) 0.1371	0.3571 (-0.01; 0.73) 0.0575
Gastrointestinal disorders, n (%) ^a	6 (85.7)	6 (42.9)	0.50 (0.25; 0.98)	0.13 (0.01; 1.33)	-0.4286 (-0.80; -0.06)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.2.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Ethnicity: Non-White for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=7)	Vosoritide (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.0445	0.0851	0.0219
Vomiting	4 (57.1)	1 (7.1)	0.13 (0.02; 0.92)	0.06 (0.00; 0.72)	-0.5000 (-0.89; -0.11)
			0.0410	0.0268	0.0121

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.2.702.1
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Ethnicity: White for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=13)	Vosoritide (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	13 (100.0)	12 (100.0)	NA	NA	NA
Injection site reaction	6 (46.2)	11 (91.7)	1.99 (1.08; 3.66) 0.0278	12.83 (1.26; 130.5) 0.0310	0.4551 (0.14; 0.77) 0.0044
Injection site erythema	4 (30.8)	10 (83.3)	2.71 (1.15; 6.36) 0.0222	11.25 (1.65; 76.85) 0.0136	0.5256 (0.20; 0.85) 0.0017

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.2.702.2
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Ethnicity: Non-White for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=3)	Vosoritide (N=7)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	3 (100.0)	7 (100.0)	NA	NA	NA
Injection site reaction	0	5 (71.4)	NA	NA	0.7143
			NA	NA	(-0.05; 0.96)
			NA	NA	NA
Injection site erythema	0	4 (57.1)	NA	NA	0.5714
			NA	NA	(-0.23; 0.91)
			NA	NA	NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.3.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 24 months to <36 months for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	4 (100.0)	9 (100.0)	NA	NA	NA
Gastrointestinal disorders, n (%) ^a	4 (100.0)	7 (77.8)	0.78 (0.40; 2.12)	0 NA	-0.2222 (-0.60; 0.36)
Vomiting	3 (75.0)	2 (22.2)	0.30 (0.08; 1.14)	0.10 (0.01; 1.50)	-0.5278 (-1.03; -0.02)
			0.0767	0.0944	0.0401

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.3.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 24 months to <36 months for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Injection site reaction	2 (50.0)	7 (77.8)	1.56 (0.55; 4.40) 0.4052	3.50 (0.28; 43.16) 0.3284	0.2778 (-0.28; 0.84) 0.3312
Injection site erythema	1 (25.0)	5 (55.6)	2.22 (0.37; 13.38) 0.3833	3.75 (0.27; 51.37) 0.3223	0.3056 (-0.23; 0.84) 0.2623

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.3.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 36 months to <60 months for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	12 (100.0)	10 (100.0)	NA	NA	NA
Injection site erythema	3 (25.0)	9 (90.0)	3.60 (1.32; 9.80) 0.0122	27.00 (2.34; 311.2) 0.0082	0.6500 (0.34; 0.96) 0.0000
Injection site reaction	4 (33.3)	9 (90.0)	2.70 (1.18; 6.17) 0.0185	18.00 (1.65; 196.3) 0.0177	0.5667 (0.24; 0.89) 0.0006
Gastrointestinal disorders, n (%) ^a	8 (66.7)	4 (40.0)	0.60 (0.25; 1.42)	0.33 (0.06; 1.91)	-0.2667 (-0.67; 0.14)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.003.701.002_ae_soc_pt_age36to60_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.3.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 36 months to <60 months for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.2433	0.2169	0.1959
Vomiting	6 (50.0)	3 (30.0)	0.60 (0.20; 1.81)	0.43 (0.07; 2.50)	-0.2000 (-0.60; 0.20)
			0.3640	0.3463	0.3282

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.003.701.002_ae_soc_pt_age36to60_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.3.702.1
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum: 24 months to <36 months for
BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	4 (100.0)	9 (100.0)	NA	NA	NA
Injection site reaction	2 (50.0)	7 (77.8)	1.56 (0.55; 4.40) 0.4052	3.50 (0.28; 43.16) 0.3284	0.2778 (-0.28; 0.84) 0.3312
Injection site erythema	1 (25.0)	5 (55.6)	2.22 (0.37; 13.38) 0.3833	3.75 (0.27; 51.37) 0.3223	0.3056 (-0.23; 0.84) 0.2623

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.003.702.001_ae_soc_pt_age24to36_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.3.702.2
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum: 36 months to <60 months for
BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	12 (100.0)	10 (100.0)	NA	NA	NA
Injection site erythema	3 (25.0)	9 (90.0)	3.60 (1.32; 9.80) 0.0122	27.00 (2.34; 311.2) 0.0082	0.6500 (0.34; 0.96) 0.0000
Injection site reaction	4 (33.3)	9 (90.0)	2.70 (1.18; 6.17) 0.0185	18.00 (1.65; 196.3) 0.0177	0.5667 (0.24; 0.89) 0.0006

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.003.702.002_ae_soc_pt_age36to60_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.4.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline AGV Category: <=4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=12)	Vosoritide (N=7)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	12 (100.0)	7 (100.0)	NA	NA	NA
Injection site reaction	3 (25.0)	5 (71.4)	2.86 (0.96; 8.47) 0.0582	7.50 (0.92; 61.05) 0.0596	0.4643 (0.05; 0.88) 0.0282
Injection site erythema	2 (16.7)	4 (57.1)	3.43 (0.83; 14.16) 0.0887	6.67 (0.79; 56.22) 0.0812	0.4048 (-0.02; 0.83) 0.0607
Gastrointestinal disorders, n (%) ^a	8 (66.7)	2 (28.6)	0.43 (0.12; 1.48)	0.20 (0.03; 1.53)	-0.3810 (-0.81; 0.05)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.004.701.001_ae_soc_pt_agvle4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.4.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline AGV Category: <=4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=12)	Vosoritide (N=7)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.1797	0.1206	0.0810
Vomiting	6 (50.0)	0	0 (0.00; 0.98)	0 (0.00; 0.83)	-0.5000 (-0.79; -0.02)
			NA	NA	NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.4.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline AGV Category: >4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=20)	Vosoritide (N=36)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	20 (100.0)	36 (100.0)	NA	NA	NA
Injection site erythema	11 (55.0)	29 (80.6)	1.46 (0.95; 2.25) 0.0803	3.39 (1.01; 11.34) 0.0475	0.2556 (0.00; 0.51) 0.0482
Injection site reaction	10 (50.0)	29 (80.6)	1.61 (1.01; 2.57) 0.0452	4.14 (1.24; 13.81) 0.0207	0.3056 (0.05; 0.56) 0.0186
Gastrointestinal disorders, n (%) ^a	18 (90.0)	23 (63.9)	0.71 (0.53; 0.94)	0.20 (0.04; 0.98)	-0.2611 (-0.47; -0.06)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.4.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline AGV Category: >4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=20)	Vosoritide (N=36)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.0188	0.0479	0.0124
Vomiting	11 (55.0)	11 (30.6)	0.56 (0.30; 1.05) 0.0684	0.36 (0.12; 1.12) 0.0766	-0.2444 (-0.51; 0.02) 0.0705

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.4.702.1
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Baseline AGV Category: <=4.5 cm/year for
BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=11)	Vosoritide (N=6)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	11 (100.0)	6 (100.0)	NA	NA	NA
Injection site reaction	3 (27.3)	5 (83.3)	3.06 (1.09; 8.55) 0.0334	13.33 (1.07; 166.4) 0.0443	0.5606 (0.16; 0.96) 0.0057
Injection site erythema	2 (18.2)	4 (66.7)	3.67 (0.93; 14.51) 0.0641	9.00 (0.91; 88.57) 0.0597	0.4848 (0.04; 0.93) 0.0311

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.4.702.2
Selected Adverse Events including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category: >4.5 cm/year for
BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=5)	Vosoritide (N=13)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	5 (100.0)	13 (100.0)	NA	NA	NA
Injection site reaction	3 (60.0)	11 (84.6)	1.41 (0.66; 2.99) 0.3704	3.67 (0.35; 38.03) 0.2763	0.2462 (-0.23; 0.72) 0.3068
Injection site erythema	2 (40.0)	10 (76.9)	1.92 (0.63; 5.86) 0.2499	5.00 (0.55; 45.39) 0.1527	0.3692 (-0.12; 0.86) 0.1370

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.5.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: <= -4 for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=18)	Vosoritide (N=21)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	18 (100.0)	21 (100.0)	NA	NA	NA
Injection site erythema	3 (16.7)	15 (71.4)	4.29 (1.47; 12.47) 0.0076	12.50 (2.63; 59.47) 0.0015	0.5476 (0.29; 0.81) 0.0000
Injection site reaction	4 (22.2)	15 (71.4)	3.21 (1.30; 7.95) 0.0115	8.75 (2.03; 37.67) 0.0036	0.4921 (0.22; 0.76) 0.0004
Gastrointestinal disorders, n (%) ^a	13 (72.2)	12 (57.1)	0.79 (0.50; 1.26)	0.51 (0.13; 1.97)	-0.1508 (-0.45; 0.15)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:10 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.005.701.001_ae_soc_pt_hazle4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.5.701.1
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: <= -4 for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=18)	Vosoritide (N=21)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.3270	0.3307	0.3180
Vomiting	8 (44.4)	6 (28.6)	0.64 (0.27; 1.51)	0.50 (0.13; 1.88)	-0.1587 (-0.46; 0.14)
			0.3088	0.3059	0.2998

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.5.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: > -4 for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=14)	Vosoritide (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	14 (100.0)	22 (100.0)	NA	NA	NA
Injection site reaction	9 (64.3)	19 (86.4)	1.34 (0.88; 2.05) 0.1726	3.52 (0.68; 18.07) 0.1319	0.2208 (-0.07; 0.51) 0.1344
Injection site erythema	10 (71.4)	18 (81.8)	1.15 (0.78; 1.68) 0.4898	1.80 (0.37; 8.80) 0.4679	0.1039 (-0.18; 0.39) 0.4769
Gastrointestinal disorders, n (%) ^a	13 (92.9)	13 (59.1)	0.64 (0.44; 0.93)	0.11 (0.01; 1.01)	-0.3377 (-0.58; -0.09)

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.5.701.2
Selected Adverse Events including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: > -4 for BMN111-206
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=14)	Vosoritide (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
			0.0187	0.0507	0.0071
Vomiting	9 (64.3)	5 (22.7)	0.35 (0.15; 0.84)	0.16 (0.04; 0.72)	-0.4156 (-0.72; -0.11)
			0.0183	0.0164	0.0078

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_206.sas, Database: N/A

Table 14.3.1.6.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Sex: Male for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	Vosoritide (N=25)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	6 (46.2)	23 (92.0)	1.99 (1.10; 3.63) 0.0239	13.42 (2.20; 82.00) 0.0049	0.4585 (0.17; 0.75) 0.0020
Hypersensitivity (SMQ Narrow Terms)	5 (38.5)	9 (36.0)	0.94 (0.39; 2.22) 0.8807	0.90 (0.23; 3.59) 0.8814	-0.0246 (-0.35; 0.30) 0.8818

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.006.701.001_ae_sbj_eoi_male_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.6.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Sex: Male for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	Vosoritide (N=25)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.006.701.001_ae_sbj_eoi_male_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.6.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Sex: Female for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=19)	Vosoritide (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	11 (57.9)	14 (77.8)	1.34 (0.85; 2.12) 0.2045	2.55 (0.61; 10.71) 0.2025	0.1988 (-0.09; 0.49) 0.1843
Hypersensitivity (SMQ Narrow Terms)	6 (31.6)	8 (44.4)	1.41 (0.61; 3.26) 0.4250	1.73 (0.45; 6.63) 0.4217	0.1287 (-0.18; 0.44) 0.4167

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.006.701.002_ae_sbj_eoi_female_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.6.701.2
 Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Sex: Female for BMN111-206
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=19)	Vosoritide (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.006.701.002_ae_sbj_eoi_female_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.6.702.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Sex: Male for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=7)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	4 (57.1)	10 (100.0)	1.75 (1.04; 7.62) NA	NA NA NA	0.4286 (0.03; 0.82) NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.006.702.001_ae_sbj_eoi_male_c1_206_saf.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.6.702.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Sex: Female for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=9)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	3 (33.3)	6 (66.7)	2.00 (0.71; 5.62) 0.1885	4.00 (0.56; 28.40) 0.1657	0.3333 (-0.10; 0.77) 0.1336

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.006.702.002_ae_sbj_eoi_female_c1_206_saf.pdf+rftf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.7.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Ethnicity: White for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=25)	Vosoritide (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	16 (64.0)	27 (93.1)	1.45 (1.07; 1.98) 0.0179	7.59 (1.46; 39.63) 0.0162	0.2910 (0.08; 0.50) 0.0065
Hypersensitivity (SMQ Narrow Terms)	6 (24.0)	12 (41.4)	1.72 (0.76; 3.92) 0.1935	2.24 (0.69; 7.26) 0.1809	0.1738 (-0.07; 0.42) 0.1649

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.007.701.001_ae_sbj_eoi_white_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.7.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Ethnicity: White for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=25)	Vosoritide (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.007.701.001_ae_sbj_eoi_white_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.7.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Ethnicity: Non-White for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=7)	Vosoritide (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	1 (14.3)	10 (71.4)	5.00 (0.79; 31.63) 0.0872	15.00 (1.34; 167.6) 0.0279	0.5714 (0.22; 0.92) 0.0014
Hypersensitivity (SMQ Narrow Terms)	5 (71.4)	5 (35.7)	0.50 (0.21; 1.16) 0.1077	0.22 (0.03; 1.59) 0.1347	-0.3571 (-0.78; 0.06) 0.0943

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.007.701.002_ae_sbj_eoi_nonwhite_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.7.701.2
 Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Ethnicity: Non-White for BMN111-206
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=7)	Vosoritide (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.007.701.002_ae_sbj_eoi_nonwhite_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.7.702.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Ethnicity: White for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	Vosoritide (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	7 (53.8)	11 (91.7)	1.70 (1.00; 2.90) 0.0497	9.43 (0.93; 95.89) 0.0580	0.3782 (0.07; 0.69) 0.0178

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.007.702.001_ae_sbj_eoi_white_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.7.702.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Ethnicity: Non-White for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=3)	Vosoritide (N=7)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	0	5 (71.4)	NA	NA	0.7143
			NA	NA	(-0.05; 0.96)
			NA	NA	NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.007.702.002_ae_sbj_eoi_nonwhite_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 24 months to <36 months for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	2 (50.0)	7 (77.8)	1.56 (0.55; 4.40) 0.4052	3.50 (0.28; 43.16) 0.3284	0.2778 (-0.28; 0.84) 0.3312
Hypersensitivity (SMQ Narrow Terms)	2 (50.0)	2 (22.2)	0.44 (0.09; 2.13) 0.3103	0.29 (0.02; 3.52) 0.3284	-0.2778 (-0.84; 0.28) 0.3312

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.701.001_ae_sbj_eoi_age24to36_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.701.1

Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 24 months to <36 months for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.701.001_ae_sbj_eoi_age24to36_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 36 months to <60 months for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	5 (41.7)	9 (90.0)	2.16 (1.07; 4.35) 0.0312	12.60 (1.19; 133.9) 0.0356	0.4833 (0.15; 0.82) 0.0047
Hypersensitivity (SMQ Narrow Terms)	3 (25.0)	5 (50.0)	2.00 (0.63; 6.38) 0.2413	3.00 (0.50; 18.17) 0.2319	0.2500 (-0.15; 0.65) 0.2148

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.701.002_ae_sbj_eoi_age36to60_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.701.2

Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Cohort 1 Age Stratum: 36 months to <60 months for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.701.002_ae_sbj_eoi_age36to60_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.702.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum: 24 months to <36 months for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	2 (50.0)	7 (77.8)	1.56 (0.55; 4.40) 0.4052	3.50 (0.28; 43.16) 0.3284	0.2778 (-0.28; 0.84) 0.3312

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.702.001_ae_sbj_eoi_age24to36_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.702.1

Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum: 24 months to < 36 months for BMN111-206

Analysis Population: Safety Analysis Set

AE Category	Placebo (N=4)	Vosoritide (N=9)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.702.001_ae_sbj_eoi_age24to36_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.702.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (>= 24 to < 60 months)) by Cohort 1 Age Stratum: 36 months to <60 months for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	5 (41.7)	9 (90.0)	2.16 (1.07; 4.35) 0.0312	12.60 (1.19; 133.9) 0.0356	0.4833 (0.15; 0.82) 0.0047

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.702.002_ae_sbj_eoi_age36to60_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.8.702.2

Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Cohort 1 Age Stratum: 36 months to < 60 months for BMN111-206

Analysis Population: Safety Analysis Set

AE Category	Placebo (N=12)	Vosoritide (N=10)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.008.702.002_ae_sbj_eoi_age36to60_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline AGV Category: <=4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=12)	Vosoritide (N=7)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	4 (33.3)	6 (85.7)	2.57 (1.09; 6.05) 0.0305	12.00 (1.05; 136.8) 0.0454	0.5238 (0.15; 0.90) 0.0058
Hypersensitivity (SMQ Narrow Terms)	2 (16.7)	3 (42.9)	2.57 (0.56; 11.84) 0.2255	3.75 (0.44; 31.62) 0.2243	0.2619 (-0.16; 0.68) 0.2248

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.701.001_ae_sbj_eoi_agvle4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.701.1
 Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline AGV Category: ≤ 4.5 cm/year for BMN111-206
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=12)	Vosoritide (N=7)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.701.001_ae_sbj_eoi_agvle4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline AGV Category: >4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=20)	Vosoritide (N=36)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	13 (65.0)	31 (86.1)	1.32 (0.94; 1.87) 0.1125	3.34 (0.89; 12.47) 0.0730	0.2111 (-0.03; 0.45) 0.0816
Hypersensitivity (SMQ Narrow Terms)	9 (45.0)	14 (38.9)	0.86 (0.46; 1.63) 0.6520	0.78 (0.26; 2.35) 0.6563	-0.0611 (-0.33; 0.21) 0.6573

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.701.002_ae_sbj_eoi_agvgt4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline AGV Category: >4.5 cm/year for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=20)	Vosoritide (N=36)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.701.002_ae_sbj_eoi_agvgt4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.702.1

Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category: ≤ 4.5 cm/year for BMN111-206

Analysis Population: Safety Analysis Set

AE Category	Placebo (N=11)	Vosoritide (N=6)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	4 (36.4)	5 (83.3)	2.29 (0.97; 5.41) 0.0587	8.75 (0.74; 103.8) 0.0857	0.4697 (0.06; 0.88) 0.0255

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.702.001_ae_sbj_eoi_agvle4_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.702.1

Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category: ≤ 4.5 cm/year for BMN111-206

Analysis Population: Safety Analysis Set

AE Category	Placebo (N=11)	Vosoritide (N=6)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.702.001_ae_sbj_eoi_agvle4_c1_206_saf.pdf+rftf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.702.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category: >4.5 cm/year for
BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=5)	Vosoritide (N=13)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	3 (60.0)	11 (84.6)	1.41 (0.66; 2.99) 0.3704	3.67 (0.35; 38.03) 0.2763	0.2462 (-0.23; 0.72) 0.3068

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.702.002_ae_sbj_eoi_agvgt4_c1_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.9.702.2

Selected Adverse Events of Interest including Treatment Group Comparisons (Cohort 1 (≥ 24 to < 60 months)) by Baseline AGV Category: >4.5 cm/year for BMN111-206

Analysis Population: Safety Analysis Set

AE Category	Placebo (N=5)	Vosoritide (N=13)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.10.701.1
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: <= -4 for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=18)	Vosoritide (N=21)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	7 (38.9)	16 (76.2)	1.96 (1.05; 3.67) 0.0354	5.03 (1.26; 20.00) 0.0219	0.3730 (0.08; 0.66) 0.0116
Hypersensitivity (SMQ Narrow Terms)	6 (33.3)	9 (42.9)	1.29 (0.57; 2.92) 0.5475	1.50 (0.41; 5.54) 0.5431	0.0952 (-0.21; 0.40) 0.5388

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.701.001_ae_sbj_eoi_hazle4_ov_206_saf.pdf+rftf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.10.701.1
 Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: ≤ -4 for BMN111-206
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=18)	Vosoritide (N=21)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.701.001_ae_sbj_eoi_hazle4_ov_206_saf.pdf+rftf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.10.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: > -4 for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=14)	Vosoritide (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	10 (71.4)	21 (95.5)	1.34 (0.95; 1.88) 0.0982	8.40 (0.83; 85.23) 0.0718	0.2403 (-0.01; 0.49) 0.0618
Hypersensitivity (SMQ Narrow Terms)	5 (35.7)	8 (36.4)	1.02 (0.42; 2.49) 0.9685	1.03 (0.25; 4.16) 0.9685	0.0065 (-0.32; 0.33) 0.9684

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 21JUN2023 09:06 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.701.002_ae_sbj_eoi_hazgt4_ov_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.1.10.701.2
Selected Adverse Events of Interest including Treatment Group Comparisons (Overall) by Baseline Height Z-score Category: > -4 for BMN111-206
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=14)	Vosoritide (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_206.sas, Database: N/A

Table 14.3.2.101.1
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing Any Adverse Event (Overall) for BMN111-206
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	NA
Race*Treatment Interaction	NA
Cohort 1 age stratum *Treatment Interaction	NA
Baseline AGV Category*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	NA

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.001.000_ae_aae_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.101.2
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event (Overall): Injection Site Reaction for BMN111-206
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.8008
Race*Treatment Interaction	0.5499
Cohort 1 age stratum *Treatment Interaction	0.4160
Baseline AGV Category*Treatment Interaction	0.3422
Baseline Height Z-Score Category*Treatment Interaction	0.0873

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.002.000_ae_isr_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.101.3
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event (Overall): Injection site erythema for BMN111-206
Analysis Population: Safety Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.6856
Race*Treatment Interaction	0.4137
Cohort 1 age stratum *Treatment Interaction	0.6455
Baseline AGV Category*Treatment Interaction	0.2605
Baseline Height Z-Score Category*Treatment Interaction	0.0227

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.003.000_ae_ise_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.101.4
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event (Overall): Gastrointestinal disorders (SOC) for
BMN111-206
Analysis Population: Safety Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.9807
Race*Treatment Interaction	0.1984
Cohort 1 age stratum *Treatment Interaction	0.5830
Baseline AGV Category*Treatment Interaction	0.4362
Baseline Height Z-Score Category*Treatment Interaction	0.4776

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.004.000_ae_gsd_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.3.2.101.5
Subgroup* Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event (Overall): Vomiting for BMN111-206
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex* Treatment Interaction	0.7451
Race* Treatment Interaction	0.1178
Cohort 1 age stratum * Treatment Interaction	0.4270
Baseline AGV Category* Treatment Interaction	NE
Baseline Height Z-Score Category* Treatment Interaction	0.3338

NA, not applicable or not presented; NE, not estimable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 21JUN2023 08:39 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.101.005.000_ae_vom_ov_int_pval_sub_206_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_206.sas, Database: N/A

Table 14.2.1.7.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	31
Mean (SD)	3.85 (1.15)	4.02 (1.78)
Median	3.93	3.97
25th, 75th Percentile	3.11, 4.57	2.97, 5.42
Min, Max	1.5, 5.9	-0.1, 6.9
Week 52		
n	33	31
Mean (SD)	4.12 (0.94)	5.53 (1.26)
Median	4.10	5.74
25th, 75th Percentile	3.58, 4.63	4.97, 6.37

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.007.001.000_mod_sub_sex_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.7.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.4, 6.5	2.3, 8.4
Change from baseline		
n	33	31
Mean (SD)	0.27 (1.68)	1.51 (1.90)
Median	0.18	1.98
25th, 75th Percentile	-0.93, 0.89	-0.12, 2.77
Min, Max	-2.4, 4.5	-1.9, 6.5
LS mean change from baseline (95% CI)	0.82 (0.36, 1.27)	2.17 (1.69, 2.65)
Difference in LS mean change from baseline (95% CI) ^a		1.36 (0.88, 1.83)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.007.001.000_mod_sub_sex_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.7.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
P-value ^b		<.0001
SMD (95% CI) ^c		1.44 (0.88, 1.99)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.007.001.000_mod_sub_sex_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.7.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	29
Mean (SD)	4.31 (1.24)	4.52 (1.20)
Median	4.39	4.57
25th, 75th Percentile	3.58, 5.20	3.71, 5.51
Min, Max	1.5, 6.7	2.5, 6.6
Week 52		
n	28	29
Mean (SD)	3.73 (1.19)	5.70 (0.79)
Median	3.56	5.80
25th, 75th Percentile	3.04, 4.66	5.29, 6.28

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.007.001.000_mod_sub_sex_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.7.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 5.9	4.2, 7.1
Change from baseline		
n	28	29
Mean (SD)	-0.58 (1.72)	1.18 (1.49)
Median	-0.76	1.17
25th, 75th Percentile	-1.72, 0.60	-0.11, 2.31
Min, Max	-3.6, 3.5	-2.1, 4.1
LS mean change from baseline (95% CI)	-0.65 (-1.08, -0.22)	1.26 (0.88, 1.65)
Difference in LS mean change from baseline (95% CI) ^a		1.91 (1.37, 2.46)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.7.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
P-value ^b		<.0001
SMD (95% CI) ^c		1.94 (1.28, 2.60)
P-value for interaction term,treatment * [Sex]		0.2562

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Baseline		
n	24	31
Mean (SD)	4.29 (1.43)	4.31 (1.61)
Median	4.56	4.26
25th, 75th Percentile	3.75, 5.31	3.08, 5.51
Min, Max	1.5, 6.7	-0.1, 6.9
Week 52		
n	24	31
Mean (SD)	4.32 (1.03)	5.76 (0.74)
Median	4.46	5.75
25th, 75th Percentile	3.62, 4.93	5.31, 6.37

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.2, 6.5	4.4, 6.9
Change from baseline		
n	24	31
Mean (SD)	0.04 (2.11)	1.45 (1.95)
Median	-0.55	1.39
25th, 75th Percentile	-1.46, 0.90	-0.18, 2.58
Min, Max	-3.6, 4.5	-2.1, 6.5
LS mean change from baseline (95% CI)	0.07 (-0.25, 0.40)	1.43 (1.14, 1.71)
Difference in LS mean change from baseline (95% CI) ^a		1.35 (0.91, 1.79)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.71 (1.06, 2.34)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	17
Mean (SD)	4.04 (1.06)	4.34 (1.18)
Median	4.02	4.06
25th, 75th Percentile	3.41, 4.86	3.41, 5.59
Min, Max	2.1, 5.8	2.5, 6.5
Week 52		
n	24	17
Mean (SD)	3.61 (0.93)	5.74 (0.83)
Median	3.60	5.86
25th, 75th Percentile	2.95, 4.29	5.37, 6.29

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 5.2	4.0, 6.8
Change from baseline		
n	24	17
Mean (SD)	-0.43 (1.49)	1.40 (1.50)
Median	-0.77	1.77
25th, 75th Percentile	-1.63, 0.64	0.21, 2.39
Min, Max	-2.7, 2.9	-1.9, 3.6
LS mean change from baseline (95% CI)	-0.48 (-1.11, 0.16)	1.84 (1.24, 2.44)
Difference in LS mean change from baseline (95% CI) ^a		2.32 (1.67, 2.96)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		2.59 (1.64, 3.50)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.008.001.000_mod_sub_age_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	12
Mean (SD)	3.68 (0.96)	4.02 (1.84)
Median	3.84	4.27
25th, 75th Percentile	2.88, 3.93	2.77, 5.28
Min, Max	2.4, 6.0	-0.1, 6.8
Week 52		
n	13	12
Mean (SD)	3.85 (1.25)	5.05 (1.74)
Median	4.10	4.99
25th, 75th Percentile	2.99, 4.63	3.90, 6.07

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.008.001.000_mod_sub_age_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.7, 5.7	2.3, 8.4
Change from baseline		
n	13	12
Mean (SD)	0.17 (1.42)	1.03 (1.36)
Median	-0.12	1.16
25th, 75th Percentile	-0.47, 0.77	-0.29, 2.30
Min, Max	-3.0, 2.7	-0.6, 2.9
LS mean change from baseline (95% CI)	0.15 (-0.60, 0.90)	0.93 (-0.03, 1.88)
Difference in LS mean change from baseline (95% CI) ^a		0.77 (-0.53, 2.08)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.008.001.000_mod_sub_age_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.8.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2266
SMD (95% CI) ^c		0.65 (-0.40, 1.68)
P-value for interaction term, treatment * [Age at Baseline]		0.0310

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.9.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	48
Mean (SD)	4.20 (1.24)	4.02 (1.55)
Median	4.47	3.97
25th, 75th Percentile	3.44, 4.99	3.00, 5.18
Min, Max	1.5, 6.7	-0.1, 6.9
Week 52		
n	48	48
Mean (SD)	3.99 (0.99)	5.51 (1.04)
Median	3.90	5.67
25th, 75th Percentile	3.16, 4.63	4.85, 6.28

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.9.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.2, 6.5	2.3, 6.9
Change from baseline		
n	48	48
Mean (SD)	-0.21 (1.87)	1.48 (1.80)
Median	-0.55	1.82
25th, 75th Percentile	-1.57, 0.75	-0.08, 2.59
Min, Max	-3.6, 4.5	-2.1, 6.5
LS mean change from baseline (95% CI)	-0.04 (-0.30, 0.23)	1.34 (1.07, 1.60)
Difference in LS mean change from baseline (95% CI) ^a		1.38 (1.00, 1.75)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.9.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.52 (1.05, 1.97)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.9.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	12
Mean (SD)	3.56 (0.94)	5.21 (1.01)
Median	3.53	5.28
25th, 75th Percentile	2.72, 3.93	4.54, 5.96
Min, Max	2.4, 5.7	3.7, 6.8
Week 52		
n	13	12
Mean (SD)	3.76 (1.36)	6.04 (1.04)
Median	4.31	6.06
25th, 75th Percentile	2.41, 4.63	5.34, 6.39

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.9.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 5.4	4.5, 8.4
Change from baseline		
n	13	12
Mean (SD)	0.21 (1.14)	0.82 (1.17)
Median	0.06	0.69
25th, 75th Percentile	-0.51, 0.70	-0.19, 1.66
Min, Max	-1.4, 2.7	-0.7, 2.7
LS mean change from baseline (95% CI)	-0.13 (-0.86, 0.60)	1.34 (0.52, 2.16)
Difference in LS mean change from baseline (95% CI) ^a		1.47 (0.25, 2.68)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.9.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0206
SMD (95% CI) ^c		1.39 (0.21, 2.54)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.0419

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	28
Mean (SD)	3.93 (1.19)	3.80 (1.72)
Median	4.11	3.87
25th, 75th Percentile	3.25, 4.73	2.90, 4.96
Min, Max	1.5, 5.9	-0.1, 6.9
Week 52		
n	28	28
Mean (SD)	4.12 (0.93)	5.41 (1.19)
Median	4.04	5.66
25th, 75th Percentile	3.51, 4.63	4.83, 6.33

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.6, 6.5	2.3, 6.9
Change from baseline		
n	28	28
Mean (SD)	0.19 (1.76)	1.62 (1.95)
Median	0.07	2.06
25th, 75th Percentile	-1.12, 0.90	-0.08, 2.82
Min, Max	-2.4, 4.5	-1.9, 6.5
LS mean change from baseline (95% CI)	0.27 (-0.07, 0.61)	1.54 (1.19, 1.88)
Difference in LS mean change from baseline (95% CI) ^a		1.27 (0.78, 1.76)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
P-value ^b		<.0001
SMD (95% CI) ^c		1.40 (0.80, 1.98)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	4.57 (1.23)	4.33 (1.27)
Median	4.71	4.10
25th, 75th Percentile	4.01, 5.38	3.08, 5.34
Min, Max	1.5, 6.7	2.5, 6.6
Week 52		
n	20	20
Mean (SD)	3.81 (1.07)	5.63 (0.80)
Median	3.55	5.67
25th, 75th Percentile	3.12, 4.63	5.01, 6.20

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.010.001.000_mod_sub_strata_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.2, 5.9	4.2, 6.8
Change from baseline		
n	20	20
Mean (SD)	-0.76 (1.92)	1.30 (1.60)
Median	-1.14	1.30
25th, 75th Percentile	-2.06, 0.17	0.38, 2.38
Min, Max	-3.6, 3.5	-2.1, 4.1
LS mean change from baseline (95% CI)	-0.52 (-0.92, -0.11)	1.05 (0.65, 1.46)
Difference in LS mean change from baseline (95% CI) ^a		1.57 (0.98, 2.17)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.83 (1.03, 2.61)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	3.43 (0.83)	6.06 (0.73)
Median	3.53	6.03
25th, 75th Percentile	2.68, 3.93	5.35, 6.81
Min, Max	2.5, 4.5	5.4, 6.8
Week 52		
n	5	3
Mean (SD)	4.17 (1.10)	6.57 (1.66)
Median	4.31	6.27
25th, 75th Percentile	4.10, 4.63	5.09, 8.37

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.4, 5.4	5.1, 8.4
Change from baseline		
n	5	3
Mean (SD)	0.74 (1.21)	0.51 (0.94)
Median	0.70	0.24
25th, 75th Percentile	-0.12, 0.77	-0.27, 1.56
Min, Max	-0.4, 2.7	-0.3, 1.6
LS mean change from baseline (95% CI)	0.37 (-2.68, 3.41)	1.13 (-3.52, 5.78)
Difference in LS mean change from baseline (95% CI) ^a		0.76 (-6.27, 7.80)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.7526
SMD (95% CI) ^c		0.56 (-2.71, 3.75)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	9
Mean (SD)	3.64 (1.05)	4.93 (0.95)
Median	3.58	4.66
25th, 75th Percentile	2.80, 4.11	4.51, 5.59
Min, Max	2.4, 5.7	3.7, 6.5
Week 52		
n	8	9
Mean (SD)	3.51 (1.52)	5.86 (0.80)
Median	3.98	5.86
25th, 75th Percentile	2.05, 4.75	5.59, 6.34

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 5.2	4.5, 7.1
Change from baseline		
n	8	9
Mean (SD)	-0.12 (1.03)	0.93 (1.27)
Median	-0.22	1.14
25th, 75th Percentile	-0.98, 0.64	-0.11, 1.77
Min, Max	-1.4, 1.5	-0.7, 2.7
LS mean change from baseline (95% CI)	-0.44 (-1.34, 0.46)	1.21 (0.37, 2.05)
Difference in LS mean change from baseline (95% CI) ^a		1.65 (0.31, 3.00)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.10.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0199
SMD (95% CI) ^c		1.58 (0.24, 2.87)
P-value for interaction term, treatment * [Strata]		0.2055

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	15
Mean (SD)	3.80 (1.40)	4.22 (1.21)
Median	3.76	4.14
25th, 75th Percentile	2.88, 4.97	3.01, 4.89
Min, Max	1.5, 5.6	2.5, 6.6
Week 52		
n	10	15
Mean (SD)	3.39 (1.00)	5.09 (1.26)
Median	3.53	5.45
25th, 75th Percentile	2.37, 4.11	4.19, 6.10

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.2, 5.0	2.3, 6.8
Change from baseline		
n	10	15
Mean (SD)	-0.41 (1.95)	0.87 (1.69)
Median	-0.96	1.15
25th, 75th Percentile	-1.94, 0.58	-0.50, 2.44
Min, Max	-2.3, 3.5	-2.1, 3.6
LS mean change from baseline (95% CI)	-0.51 (-1.37, 0.34)	1.17 (0.50, 1.85)
Difference in LS mean change from baseline (95% CI) ^a		1.69 (0.70, 2.68)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0021
SMD (95% CI) ^c		1.66 (0.59, 2.70)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	3.81 (1.24)	3.86 (1.74)
Median	3.90	3.99
25th, 75th Percentile	2.70, 4.65	3.39, 5.17
Min, Max	1.5, 6.7	-0.1, 6.5
Week 52		
n	24	18
Mean (SD)	4.10 (1.09)	5.37 (0.93)
Median	4.08	5.42
25th, 75th Percentile	3.47, 4.82	4.73, 6.28

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.7, 6.0	2.8, 6.5
Change from baseline		
n	24	18
Mean (SD)	0.29 (1.73)	1.52 (1.82)
Median	-0.18	1.75
25th, 75th Percentile	-0.61, 1.08	0.21, 2.39
Min, Max	-3.6, 4.5	-1.3, 6.5
LS mean change from baseline (95% CI)	0.38 (-0.17, 0.93)	1.52 (0.90, 2.14)
Difference in LS mean change from baseline (95% CI) ^a		1.14 (0.43, 1.84)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.011.001.000_mod_sub_bhgt_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0024
SMD (95% CI) ^c		1.09 (0.38, 1.79)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	4.42 (1.05)	4.49 (1.53)
Median	4.51	4.43
25th, 75th Percentile	3.51, 5.24	3.11, 5.88
Min, Max	2.2, 6.0	1.6, 6.9
Week 52		
n	19	22
Mean (SD)	3.87 (1.04)	5.98 (0.75)
Median	3.78	6.14
25th, 75th Percentile	3.11, 4.78	5.59, 6.59

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 5.2	4.4, 7.1
Change from baseline		
n	19	22
Mean (SD)	-0.55 (1.55)	1.49 (1.75)
Median	-0.93	1.58
25th, 75th Percentile	-1.64, 0.58	-0.12, 2.61
Min, Max	-3.0, 2.9	-1.7, 4.1
LS mean change from baseline (95% CI)	-0.51 (-1.18, 0.16)	1.58 (1.04, 2.11)
Difference in LS mean change from baseline (95% CI) ^a		2.09 (1.43, 2.75)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		2.19 (1.33, 3.02)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	4.28 (1.14)	4.80 (1.69)
Median	4.22	4.66
25th, 75th Percentile	3.52, 5.16	3.71, 6.12
Min, Max	2.5, 5.9	2.7, 6.8
Week 52		
n	8	5
Mean (SD)	4.33 (1.10)	6.40 (1.15)
Median	4.24	5.94
25th, 75th Percentile	3.64, 4.80	5.80, 6.45

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.8, 6.5	5.5, 8.4
Change from baseline		
n	8	5
Mean (SD)	0.05 (1.95)	1.60 (1.23)
Median	-0.02	1.56
25th, 75th Percentile	-1.54, 0.80	1.14, 2.74
Min, Max	-2.0, 3.9	-0.2, 2.8
LS mean change from baseline (95% CI)	-0.64 (-2.09, 0.81)	2.26 (1.26, 3.25)
Difference in LS mean change from baseline (95% CI) ^a		2.90 (0.93, 4.86)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.11.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0128
SMD (95% CI) ^c		3.97 (0.75, 7.05)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.3158

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	19
Mean (SD)	2.64 (0.67)	2.56 (1.03)
Median	2.68	2.97
25th, 75th Percentile	2.23, 3.39	2.55, 3.09
Min, Max	1.5, 3.5	-0.1, 3.4
Week 52		
n	19	19
Mean (SD)	3.98 (1.41)	5.44 (1.24)
Median	4.02	5.58
25th, 75th Percentile	2.57, 5.00	5.10, 6.37

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 6.5	2.3, 6.9
Change from baseline		
n	19	19
Mean (SD)	1.34 (1.77)	2.88 (1.38)
Median	0.90	2.77
25th, 75th Percentile	-0.12, 2.92	2.12, 3.62
Min, Max	-1.4, 4.5	-0.5, 6.5
LS mean change from baseline (95% CI)	1.69 (1.12, 2.27)	2.60 (1.86, 3.33)
Difference in LS mean change from baseline (95% CI) ^a		0.90 (0.17, 1.64)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0170
SMD (95% CI) ^c		0.92 (0.16, 1.67)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	4.03 (0.30)	3.96 (0.24)
Median	4.01	3.97
25th, 75th Percentile	3.84, 4.29	3.75, 4.14
Min, Max	3.5, 4.5	3.5, 4.4
Week 52		
n	18	14
Mean (SD)	4.09 (0.93)	5.79 (0.98)
Median	4.18	6.14
25th, 75th Percentile	3.44, 4.78	5.29, 6.45

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.2, 5.7	3.3, 6.8
Change from baseline		
n	18	14
Mean (SD)	0.06 (0.97)	1.83 (1.05)
Median	0.22	2.32
25th, 75th Percentile	-0.66, 0.66	1.15, 2.58
Min, Max	-1.9, 1.8	-0.6, 2.8
LS mean change from baseline (95% CI)	0.16 (-0.37, 0.69)	2.00 (1.38, 2.63)
Difference in LS mean change from baseline (95% CI) ^a		1.84 (1.09, 2.59)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.99 (1.03, 2.93)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.012.001.000_mod_sub_bagv_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	27
Mean (SD)	5.20 (0.58)	5.61 (0.73)
Median	5.09	5.59
25th, 75th Percentile	4.71, 5.65	4.89, 6.06
Min, Max	4.5, 6.7	4.5, 6.9
Week 52		
n	24	27
Mean (SD)	3.80 (0.89)	5.64 (0.96)
Median	3.71	5.63
25th, 75th Percentile	3.10, 4.46	4.89, 6.27

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.3, 5.9	4.0, 8.4
Change from baseline		
n	24	27
Mean (SD)	-1.40 (1.11)	0.03 (1.05)
Median	-1.49	-0.11
25th, 75th Percentile	-2.10, -0.74	-0.65, 1.10
Min, Max	-3.6, 1.2	-2.1, 1.8
LS mean change from baseline (95% CI)	-0.89 (-1.44, -0.34)	0.53 (0.15, 0.92)
Difference in LS mean change from baseline (95% CI) ^a		1.42 (0.86, 1.98)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.12.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.72 (0.96, 2.45)
P-value for interaction term,treatment * [Baseline AGV]		0.8219

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.13.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	45
Mean (SD)	3.95 (1.17)	4.06 (1.63)
Median	4.09	3.98
25th, 75th Percentile	3.39, 4.72	2.99, 5.20
Min, Max	1.5, 6.0	-0.1, 6.9
Week 52		
n	41	45
Mean (SD)	3.84 (1.19)	5.58 (1.13)
Median	3.78	5.75
25th, 75th Percentile	3.11, 4.77	4.89, 6.34

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.13.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 6.5	2.3, 8.4
Change from baseline		
n	41	45
Mean (SD)	-0.12 (1.77)	1.52 (1.72)
Median	-0.31	1.65
25th, 75th Percentile	-1.46, 0.62	-0.11, 2.59
Min, Max	-3.0, 4.5	-1.7, 6.5
LS mean change from baseline (95% CI)	0.12 (-0.31, 0.55)	1.73 (1.32, 2.14)
Difference in LS mean change from baseline (95% CI) ^a		1.61 (1.14, 2.08)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.13.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.50 (1.01, 1.99)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.13.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	15
Mean (SD)	4.28 (1.27)	4.85 (1.04)
Median	4.39	4.89
25th, 75th Percentile	3.76, 5.07	3.83, 5.59
Min, Max	1.5, 6.7	3.1, 6.6
Week 52		
n	20	15
Mean (SD)	4.16 (0.75)	5.71 (0.78)
Median	4.35	5.63
25th, 75th Percentile	3.67, 4.59	5.29, 6.28

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.1.13.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.8, 5.4	4.0, 6.9
Change from baseline		
n	20	15
Mean (SD)	-0.12 (1.73)	0.86 (1.63)
Median	-0.60	1.15
25th, 75th Percentile	-1.24, 0.80	-0.27, 2.01
Min, Max	-3.6, 3.5	-2.1, 3.8
LS mean change from baseline (95% CI)	-0.15 (-0.46, 0.15)	1.54 (1.23, 1.85)
Difference in LS mean change from baseline (95% CI) ^a		1.69 (1.32, 2.06)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.13.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
P-value ^b		<.0001
SMD (95% CI) ^c		3.42 (2.25, 4.56)
P-value for interaction term,treatment * [Ethnicity]		0.8672

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
North America		
Baseline		
n	26	27
Mean (SD)	4.02 (1.11)	3.96 (1.67)
Median	3.90	3.83
25th, 75th Percentile	3.41, 4.87	3.11, 5.17
Min, Max	1.5, 6.0	-0.1, 6.9
Week 52		
n	26	27
Mean (SD)	3.96 (0.84)	5.67 (0.91)
Median	3.90	5.82
25th, 75th Percentile	3.44, 4.48	5.10, 6.29

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.4, 5.9	2.8, 6.9
Change from baseline		
n	26	27
Mean (SD)	-0.06 (1.39)	1.70 (1.80)
Median	-0.17	2.01
25th, 75th Percentile	-0.93, 0.89	-0.03, 2.88
Min, Max	-3.0, 3.4	-0.7, 6.5
LS mean change from baseline (95% CI)	0.44 (-0.02, 0.89)	1.80 (1.43, 2.18)
Difference in LS mean change from baseline (95% CI) ^a		1.37 (0.88, 1.85)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		<.0001
SMD (95% CI) ^c		1.70 (1.01, 2.37)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
Europe		
Baseline		
n	18	18
Mean (SD)	3.96 (1.41)	4.13 (1.62)
Median	4.38	3.61
25th, 75th Percentile	2.71, 5.01	2.83, 5.88
Min, Max	1.5, 5.9	1.6, 6.8
Week 52		
n	18	18
Mean (SD)	3.80 (1.46)	5.44 (1.32)
Median	3.77	5.46
25th, 75th Percentile	2.77, 4.77	4.68, 6.28

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.01.014.001.000_mod_sub_reg_agv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	1.3, 6.5	2.3, 8.4
Change from baseline		
n	18	18
Mean (SD)	-0.16 (2.27)	1.32 (1.77)
Median	-1.12	1.60
25th, 75th Percentile	-1.81, 1.84	-0.31, 2.58
Min, Max	-2.4, 4.5	-1.9, 4.1
LS mean change from baseline (95% CI)	0.17 (-0.49, 0.83)	1.96 (1.22, 2.70)
Difference in LS mean change from baseline (95% CI) ^a		1.80 (1.06, 2.53)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
P-value ^b		<.0001
SMD (95% CI) ^c		1.70 (0.89, 2.49)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	3
Mean (SD)	4.92 (1.21)	5.71 (0.88)
Median	4.54	5.59
25th, 75th Percentile	4.20, 5.64	4.89, 6.64
Min, Max	3.9, 6.7	4.9, 6.6
Week 52		
n	4	3
Mean (SD)	3.90 (0.64)	5.42 (0.79)
Median	3.93	5.59
25th, 75th Percentile	3.45, 4.34	4.56, 6.10

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	3.1, 4.6	4.6, 6.1
Change from baseline		
n	4	3
Mean (SD)	-1.03 (1.82)	-0.29 (1.67)
Median	-0.60	0.00
25th, 75th Percentile	-2.13, 0.08	-2.08, 1.21
Min, Max	-3.6, 0.7	-2.1, 1.2
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Annualized Growth Velocity (cm/year)		
Rest of World		
Baseline		
n	13	12
Mean (SD)	4.01 (1.12)	4.76 (0.83)
Median	4.10	4.59
25th, 75th Percentile	3.39, 4.55	4.02, 5.47
Min, Max	2.2, 5.8	3.7, 6.1
Week 52		
n	13	12
Mean (SD)	4.11 (1.06)	5.78 (1.05)
Median	4.26	6.03
25th, 75th Percentile	3.58, 5.09	5.35, 6.45

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	2.2, 5.4	3.3, 7.1
Change from baseline		
n	13	12
Mean (SD)	0.10 (1.62)	1.03 (1.21)
Median	0.27	1.03
25th, 75th Percentile	-1.22, 0.62	-0.11, 2.27
Min, Max	-2.7, 2.9	-0.6, 2.8
LS mean change from baseline (95% CI)	-0.28 (-1.02, 0.47)	1.42 (0.62, 2.22)
Difference in LS mean change from baseline (95% CI) ^a		1.70 (0.71, 2.68)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.1.14.1
Analysis of Covariance of Annualized Growth Velocity (cm/year) at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Annualized Growth Velocity (cm/year)	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0021
SMD (95% CI) ^c		1.59 (0.56, 2.58)
P-value for interaction term,treatment * [Region]		0.7439

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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AGV at a Post-baseline Visit is defined as [(Height at Post-baseline Visit - Height at Baseline)/(Date of Post-baseline Visit - Date of Baseline Assessment)] x 365.25.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.7.1
Analysis of Covariance of Height Z-Score at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	31
Mean (SD)	-4.88 (0.99)	-4.89 (1.03)
Median	-5.06	-4.94
25th, 75th Percentile	-5.58, -4.24	-5.59, -4.38
Min, Max	-7.0, -2.7	-6.6, -1.1
Week 52		
n	33	31
Mean (SD)	-4.85 (0.94)	-4.68 (1.05)
Median	-4.96	-4.58
25th, 75th Percentile	-5.42, -4.36	-5.55, -4.16
Min, Max	-7.0, -2.8	-6.5, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.007.001.000_mod_sub_sex_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.7.1
Analysis of Covariance of Height Z-Score at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	33	31
Mean (SD)	0.03 (0.20)	0.21 (0.23)
Median	0.00	0.15
25th, 75th Percentile	-0.12, 0.16	0.04, 0.42
Min, Max	-0.4, 0.5	-0.2, 0.6
LS mean change from baseline (95% CI)	0.07 (-0.03, 0.17)	0.27 (0.17, 0.37)
Difference in LS mean change from baseline (95% CI) ^a		0.20 (0.10, 0.30)
P-value ^b		0.0002

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.7.1
Analysis of Covariance of Height Z-Score at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.01 (0.48, 1.54)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.2.7.1
Analysis of Covariance of Height Z-Score at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	29
Mean (SD)	-5.45 (1.10)	-5.39 (1.16)
Median	-5.34	-5.78
25th, 75th Percentile	-6.32, -4.57	-6.33, -4.46
Min, Max	-7.9, -3.6	-7.7, -3.0
Week 52		
n	28	29
Mean (SD)	-5.49 (1.16)	-5.10 (1.10)
Median	-5.24	-5.47
25th, 75th Percentile	-6.31, -4.74	-5.76, -4.24
Min, Max	-7.8, -3.4	-7.5, -2.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.7.1
Analysis of Covariance of Height Z-Score at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	29
Mean (SD)	-0.04 (0.35)	0.28 (0.39)
Median	0.05	0.31
25th, 75th Percentile	-0.29, 0.24	-0.07, 0.58
Min, Max	-0.8, 0.4	-0.4, 1.0
LS mean change from baseline (95% CI)	-0.10 (-0.27, 0.06)	0.27 (0.13, 0.42)
Difference in LS mean change from baseline (95% CI) ^a		0.38 (0.17, 0.58)
P-value ^b		0.0006

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.007.001.000_mod_sub_sex_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.7.1
Analysis of Covariance of Height Z-Score at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.02 (0.44, 1.59)
P-value for interaction term, treatment * [Sex]		0.1434

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.007.001.000_mod_sub_sex_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Baseline		
n	24	31
Mean (SD)	-5.37 (1.15)	-5.15 (0.96)
Median	-5.43	-5.02
25th, 75th Percentile	-6.04, -4.53	-5.78, -4.35
Min, Max	-7.3, -2.7	-7.7, -3.8
Week 52		
n	24	31
Mean (SD)	-5.49 (1.21)	-5.01 (0.98)
Median	-5.40	-4.76
25th, 75th Percentile	-6.37, -4.80	-5.74, -4.14
Min, Max	-7.8, -2.9	-7.5, -3.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	31
Mean (SD)	-0.12 (0.23)	0.15 (0.24)
Median	-0.11	0.13
25th, 75th Percentile	-0.23, -0.02	-0.03, 0.29
Min, Max	-0.7, 0.3	-0.2, 0.7
LS mean change from baseline (95% CI)	-0.12 (-0.21, -0.04)	0.15 (0.08, 0.22)
Difference in LS mean change from baseline (95% CI) ^a		0.27 (0.16, 0.38)
P-value ^b		<.0001

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.36 (0.75, 1.96)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	17
Mean (SD)	-4.81 (1.00)	-5.49 (0.90)
Median	-4.59	-5.42
25th, 75th Percentile	-5.53, -4.12	-6.35, -4.99
Min, Max	-6.6, -2.7	-6.6, -3.0
Week 52		
n	24	17
Mean (SD)	-4.67 (0.92)	-4.93 (0.83)
Median	-4.50	-5.03
25th, 75th Percentile	-5.24, -3.96	-5.49, -4.43
Min, Max	-6.3, -2.8	-6.5, -2.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	17
Mean (SD)	0.14 (0.18)	0.57 (0.23)
Median	0.15	0.57
25th, 75th Percentile	0.00, 0.26	0.42, 0.67
Min, Max	-0.3, 0.4	0.1, 1.0
LS mean change from baseline (95% CI)	0.20 (0.08, 0.32)	0.58 (0.47, 0.70)
Difference in LS mean change from baseline (95% CI) ^a		0.38 (0.26, 0.50)
P-value ^b		<.0001

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		2.21 (1.32, 3.07)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	12
Mean (SD)	-5.32 (0.95)	-4.55 (1.55)
Median	-5.35	-4.68
25th, 75th Percentile	-5.36, -5.01	-5.91, -3.67
Min, Max	-7.9, -3.8	-6.3, -1.1
Week 52		
n	13	12
Mean (SD)	-5.38 (0.86)	-4.52 (1.58)
Median	-5.19	-4.66
25th, 75th Percentile	-5.65, -5.11	-5.63, -3.82
Min, Max	-7.6, -3.8	-6.6, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	-0.06 (0.40)	0.03 (0.28)
Median	0.06	0.02
25th, 75th Percentile	-0.45, 0.19	-0.19, 0.22
Min, Max	-0.8, 0.5	-0.4, 0.5
LS mean change from baseline (95% CI)	-0.12 (-0.28, 0.05)	0.03 (-0.18, 0.24)
Difference in LS mean change from baseline (95% CI) ^a		0.15 (-0.14, 0.43)
P-value ^b		0.2961

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.8.1
Analysis of Covariance of Height Z-Score at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.56 (-0.48, 1.58)
P-value for interaction term, treatment * [Age at Baseline]		0.2675

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.008.001.000_mod_sub_age_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.9.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	48
Mean (SD)	-5.16 (1.07)	-5.20 (1.07)
Median	-5.22	-5.37
25th, 75th Percentile	-5.91, -4.41	-5.93, -4.44
Min, Max	-7.3, -2.7	-7.7, -1.1
Week 52		
n	48	48
Mean (SD)	-5.18 (1.11)	-4.97 (1.05)
Median	-5.10	-4.97
25th, 75th Percentile	-5.95, -4.47	-5.69, -4.18
Min, Max	-7.8, -2.8	-7.5, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.009.001.000_mod_sub_tan_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.9.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	48	48
Mean (SD)	-0.02 (0.27)	0.23 (0.28)
Median	-0.02	0.17
25th, 75th Percentile	-0.18, 0.19	0.02, 0.45
Min, Max	-0.8, 0.4	-0.2, 1.0
LS mean change from baseline (95% CI)	-0.02 (-0.10, 0.05)	0.25 (0.18, 0.33)
Difference in LS mean change from baseline (95% CI) ^a		0.27 (0.17, 0.38)
P-value ^b		<.0001

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.009.001.000_mod_sub_tan_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.9.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.06 (0.63, 1.49)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.009.001.000_mod_sub_tan_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.9.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	12
Mean (SD)	-5.06 (1.09)	-4.85 (1.30)
Median	-5.01	-4.92
25th, 75th Percentile	-5.36, -4.49	-6.01, -3.67
Min, Max	-7.9, -3.6	-6.5, -3.0
Week 52		
n	13	12
Mean (SD)	-5.00 (1.03)	-4.57 (1.22)
Median	-5.11	-4.66
25th, 75th Percentile	-5.30, -4.27	-5.54, -3.86
Min, Max	-7.6, -3.4	-6.6, -2.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.009.001.000_mod_sub_tan_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.9.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	0.06 (0.33)	0.27 (0.45)
Median	0.16	0.31
25th, 75th Percentile	0.00, 0.28	-0.19, 0.67
Min, Max	-0.6, 0.5	-0.4, 0.9
LS mean change from baseline (95% CI)	0.23 (0.03, 0.44)	0.24 (0.01, 0.47)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.33, 0.34)
P-value ^b		0.9780

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.009.001.000_mod_sub_tan_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.9.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.02 (-1.06, 1.09)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.7461

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.009.001.000_mod_sub_tan_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	28
Mean (SD)	-4.84 (1.03)	-4.95 (1.02)
Median	-4.73	-5.02
25th, 75th Percentile	-5.59, -4.23	-5.65, -4.39
Min, Max	-7.0, -2.7	-6.6, -1.1
Week 52		
n	28	28
Mean (SD)	-4.84 (1.00)	-4.78 (1.02)
Median	-4.82	-4.74
25th, 75th Percentile	-5.63, -4.26	-5.57, -4.18
Min, Max	-7.0, -2.8	-6.5, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	28
Mean (SD)	-0.01 (0.19)	0.18 (0.23)
Median	-0.02	0.13
25th, 75th Percentile	-0.14, 0.14	0.02, 0.34
Min, Max	-0.4, 0.3	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.01 (-0.08, 0.07)	0.18 (0.10, 0.25)
Difference in LS mean change from baseline (95% CI) ^a		0.18 (0.08, 0.29)
P-value ^b		0.0011

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
 Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.93 (0.37, 1.48)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	-5.62 (0.98)	-5.54 (1.06)
Median	-5.39	-5.80
25th, 75th Percentile	-6.53, -4.87	-6.33, -4.49
Min, Max	-7.3, -4.2	-7.7, -3.8
Week 52		
n	20	20
Mean (SD)	-5.66 (1.10)	-5.23 (1.06)
Median	-5.39	-5.48
25th, 75th Percentile	-6.37, -4.94	-6.00, -4.22
Min, Max	-7.8, -3.8	-7.5, -3.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	20
Mean (SD)	-0.05 (0.36)	0.31 (0.34)
Median	0.02	0.38
25th, 75th Percentile	-0.29, 0.22	0.02, 0.57
Min, Max	-0.8, 0.4	-0.2, 1.0
LS mean change from baseline (95% CI)	-0.07 (-0.22, 0.07)	0.34 (0.20, 0.49)
Difference in LS mean change from baseline (95% CI) ^a		0.42 (0.21, 0.63)
P-value ^b		0.0003

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
 Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.37 (0.62, 2.10)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	-5.12 (0.75)	-4.27 (1.12)
Median	-5.35	-4.90
25th, 75th Percentile	-5.36, -5.28	-4.94, -2.98
Min, Max	-5.8, -3.8	-4.9, -3.0
Week 52		
n	5	3
Mean (SD)	-4.90 (0.62)	-3.81 (1.14)
Median	-5.19	-4.35
25th, 75th Percentile	-5.25, -4.96	-4.58, -2.50
Min, Max	-5.3, -3.8	-4.6, -2.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	5	3
Mean (SD)	0.21 (0.21)	0.46 (0.14)
Median	0.16	0.48
25th, 75th Percentile	0.06, 0.32	0.31, 0.59
Min, Max	0.0, 0.5	0.3, 0.6
LS mean change from baseline (95% CI)	0.20 (-0.25, 0.65)	0.48 (-0.21, 1.17)
Difference in LS mean change from baseline (95% CI) ^a		0.28 (-0.76, 1.32)
P-value ^b		0.4580

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.39 (-2.08, 4.66)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	9
Mean (SD)	-5.02 (1.31)	-5.04 (1.36)
Median	-4.77	-5.68
25th, 75th Percentile	-5.32, -4.27	-6.22, -4.10
Min, Max	-7.9, -3.6	-6.5, -3.0
Week 52		
n	8	9
Mean (SD)	-5.05 (1.27)	-4.82 (1.19)
Median	-5.07	-5.03
25th, 75th Percentile	-5.48, -4.15	-5.57, -4.24
Min, Max	-7.6, -3.4	-6.6, -2.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	9
Mean (SD)	-0.03 (0.36)	0.21 (0.51)
Median	0.08	0.11
25th, 75th Percentile	-0.31, 0.25	-0.24, 0.76
Min, Max	-0.6, 0.3	-0.4, 0.9
LS mean change from baseline (95% CI)	0.10 (-0.19, 0.38)	0.10 (-0.17, 0.36)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.42, 0.42)
P-value ^b		0.9958

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.10.1
Analysis of Covariance of Height Z-Score at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.00 (-1.15, 1.16)
P-value for interaction term, treatment * [Strata]		0.3315

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.010.001.000_mod_sub_strata_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	15
Mean (SD)	-6.84 (0.52)	-6.42 (0.41)
Median	-6.77	-6.34
25th, 75th Percentile	-7.08, -6.51	-6.47, -6.14
Min, Max	-7.9, -6.1	-7.7, -6.0
Week 52		
n	10	15
Mean (SD)	-6.83 (0.65)	-6.01 (0.58)
Median	-6.74	-5.76
25th, 75th Percentile	-7.50, -6.30	-6.48, -5.58
Min, Max	-7.8, -6.1	-7.5, -5.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.011.001.000_mod_sub_bhgt_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	10	15
Mean (SD)	0.00 (0.36)	0.40 (0.40)
Median	0.10	0.45
25th, 75th Percentile	-0.20, 0.26	0.08, 0.75
Min, Max	-0.7, 0.4	-0.4, 1.0
LS mean change from baseline (95% CI)	-0.04 (-0.35, 0.28)	0.33 (0.08, 0.58)
Difference in LS mean change from baseline (95% CI) ^a		0.37 (0.00, 0.73)
P-value ^b		0.0503

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.97 (0.00, 1.92)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.011.001.000_mod_sub_bhgt_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	-5.49 (0.29)	-5.52 (0.26)
Median	-5.41	-5.53
25th, 75th Percentile	-5.71, -5.31	-5.75, -5.39
Min, Max	-6.0, -5.0	-5.8, -5.0
Week 52		
n	24	18
Mean (SD)	-5.47 (0.40)	-5.34 (0.44)
Median	-5.35	-5.41
25th, 75th Percentile	-5.72, -5.18	-5.65, -5.03
Min, Max	-6.4, -5.0	-6.0, -4.6

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.011.001.000_mod_sub_bhgt_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	18
Mean (SD)	0.02 (0.32)	0.18 (0.31)
Median	0.12	0.12
25th, 75th Percentile	-0.12, 0.22	-0.07, 0.45
Min, Max	-0.8, 0.5	-0.2, 0.8
LS mean change from baseline (95% CI)	0.00 (-0.16, 0.17)	0.23 (0.05, 0.42)
Difference in LS mean change from baseline (95% CI) ^a		0.23 (0.02, 0.44)
P-value ^b		0.0342

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.73 (0.05, 1.40)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	-4.50 (0.25)	-4.45 (0.30)
Median	-4.49	-4.43
25th, 75th Percentile	-4.66, -4.27	-4.71, -4.28
Min, Max	-4.9, -4.0	-5.0, -4.0
Week 52		
n	19	22
Mean (SD)	-4.54 (0.35)	-4.24 (0.32)
Median	-4.51	-4.22
25th, 75th Percentile	-4.82, -4.29	-4.43, -4.07
Min, Max	-5.1, -3.8	-4.8, -3.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	22
Mean (SD)	-0.05 (0.26)	0.21 (0.25)
Median	-0.05	0.19
25th, 75th Percentile	-0.27, 0.19	0.04, 0.42
Min, Max	-0.5, 0.4	-0.3, 0.6
LS mean change from baseline (95% CI)	-0.02 (-0.18, 0.15)	0.23 (0.09, 0.36)
Difference in LS mean change from baseline (95% CI) ^a		0.24 (0.08, 0.41)
P-value ^b		0.0054

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.01 (0.30, 1.72)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	-3.49 (0.51)	-2.84 (1.00)
Median	-3.70	-3.01
25th, 75th Percentile	-3.84, -3.14	-3.24, -2.98
Min, Max	-3.9, -2.7	-3.8, -1.1
Week 52		
n	8	5
Mean (SD)	-3.48 (0.42)	-2.72 (1.04)
Median	-3.59	-2.69
25th, 75th Percentile	-3.83, -3.17	-3.48, -2.50
Min, Max	-3.9, -2.8	-3.8, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.011.001.000_mod_sub_bhgt_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	5
Mean (SD)	0.00 (0.15)	0.11 (0.28)
Median	-0.01	0.01
25th, 75th Percentile	-0.09, 0.14	0.00, 0.31
Min, Max	-0.2, 0.2	-0.2, 0.5
LS mean change from baseline (95% CI)	-0.08 (-0.56, 0.39)	0.18 (-0.15, 0.51)
Difference in LS mean change from baseline (95% CI) ^a		0.27 (-0.38, 0.92)
P-value ^b		0.3388

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.11.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.11 (-1.10, 3.22)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.4818

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	19
Mean (SD)	-5.44 (1.15)	-5.18 (1.35)
Median	-5.39	-5.39
25th, 75th Percentile	-6.32, -4.66	-6.04, -4.38
Min, Max	-7.9, -3.6	-7.7, -1.1
Week 52		
n	19	19
Mean (SD)	-5.34 (1.15)	-4.91 (1.32)
Median	-5.30	-5.03
25th, 75th Percentile	-6.05, -4.48	-5.70, -4.16
Min, Max	-7.6, -3.4	-7.5, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	19
Mean (SD)	0.10 (0.26)	0.26 (0.27)
Median	0.13	0.15
25th, 75th Percentile	-0.04, 0.26	0.04, 0.55
Min, Max	-0.6, 0.5	-0.1, 0.7
LS mean change from baseline (95% CI)	0.11 (-0.02, 0.24)	0.21 (0.04, 0.38)
Difference in LS mean change from baseline (95% CI) ^a		0.10 (-0.07, 0.27)
P-value ^b		0.2281

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.012.001.000_mod_sub_bagv_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.45 (-0.28, 1.17)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	-5.01 (0.85)	-5.19 (0.97)
Median	-5.09	-5.39
25th, 75th Percentile	-5.36, -4.49	-5.83, -4.46
Min, Max	-7.1, -3.6	-6.4, -3.0
Week 52		
n	18	14
Mean (SD)	-5.04 (0.99)	-4.87 (0.94)
Median	-5.06	-4.92
25th, 75th Percentile	-5.30, -4.36	-5.69, -4.15
Min, Max	-7.8, -3.4	-6.0, -2.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	14
Mean (SD)	-0.03 (0.34)	0.33 (0.34)
Median	0.05	0.34
25th, 75th Percentile	-0.09, 0.16	0.13, 0.56
Min, Max	-0.8, 0.3	-0.3, 1.0
LS mean change from baseline (95% CI)	-0.09 (-0.29, 0.10)	0.37 (0.14, 0.61)
Difference in LS mean change from baseline (95% CI) ^a		0.47 (0.19, 0.75)
P-value ^b		0.0021

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
 Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.36 (0.49, 2.21)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	27
Mean (SD)	-4.99 (1.14)	-5.06 (1.04)
Median	-4.80	-4.94
25th, 75th Percentile	-5.94, -4.33	-6.09, -4.11
Min, Max	-7.3, -2.7	-6.6, -3.0
Week 52		
n	24	27
Mean (SD)	-5.06 (1.13)	-4.88 (1.01)
Median	-4.84	-4.58
25th, 75th Percentile	-6.01, -4.40	-5.58, -4.24
Min, Max	-7.5, -2.8	-6.6, -2.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	27
Mean (SD)	-0.07 (0.24)	0.18 (0.34)
Median	-0.10	0.11
25th, 75th Percentile	-0.23, 0.17	-0.07, 0.45
Min, Max	-0.5, 0.3	-0.4, 0.9
LS mean change from baseline (95% CI)	0.04 (-0.16, 0.23)	0.23 (0.10, 0.37)
Difference in LS mean change from baseline (95% CI) ^a		0.20 (0.00, 0.39)
P-value ^b		0.0464

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Table 14.2.2.12.1
Analysis of Covariance of Height Z-Score at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.68 (0.01, 1.35)
P-value for interaction term, treatment * [Baseline AGV]		0.2511

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.13.1
Analysis of Covariance of Height Z-Score at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	45
Mean (SD)	-5.18 (1.05)	-5.05 (1.13)
Median	-5.28	-5.19
25th, 75th Percentile	-5.58, -4.49	-5.78, -4.35
Min, Max	-7.9, -2.7	-7.7, -1.1
Week 52		
n	41	45
Mean (SD)	-5.19 (1.08)	-4.84 (1.11)
Median	-5.12	-4.81
25th, 75th Percentile	-5.65, -4.51	-5.59, -4.16
Min, Max	-7.8, -2.8	-7.5, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.13.1
Analysis of Covariance of Height Z-Score at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	41	45
Mean (SD)	-0.01 (0.29)	0.21 (0.32)
Median	0.00	0.15
25th, 75th Percentile	-0.13, 0.19	-0.02, 0.45
Min, Max	-0.8, 0.4	-0.4, 1.0
LS mean change from baseline (95% CI)	-0.04 (-0.16, 0.07)	0.22 (0.11, 0.33)
Difference in LS mean change from baseline (95% CI) ^a		0.27 (0.14, 0.40)
P-value ^b		<.0001

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.13.1
Analysis of Covariance of Height Z-Score at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.91 (0.46, 1.37)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.13.1
Analysis of Covariance of Height Z-Score at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	15
Mean (SD)	-5.06 (1.13)	-5.36 (1.06)
Median	-4.89	-5.59
25th, 75th Percentile	-5.94, -4.41	-6.35, -4.48
Min, Max	-7.1, -2.7	-6.6, -3.0
Week 52		
n	20	15
Mean (SD)	-5.05 (1.11)	-5.03 (1.03)
Median	-5.06	-5.51
25th, 75th Percentile	-5.95, -4.28	-5.74, -4.30
Min, Max	-7.2, -2.9	-6.5, -2.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.13.1
Analysis of Covariance of Height Z-Score at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	15
Mean (SD)	0.00 (0.27)	0.33 (0.32)
Median	0.05	0.31
25th, 75th Percentile	-0.22, 0.18	0.04, 0.56
Min, Max	-0.5, 0.5	-0.2, 0.9
LS mean change from baseline (95% CI)	-0.04 (-0.19, 0.11)	0.34 (0.19, 0.49)
Difference in LS mean change from baseline (95% CI) ^a		0.38 (0.20, 0.56)
P-value ^b		0.0002

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.13.1
Analysis of Covariance of Height Z-Score at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.54 (0.71, 2.35)
P-value for interaction term,treatment * [Ethnicity]		0.4489

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
North America		
Baseline		
n	26	27
Mean (SD)	-5.13 (0.97)	-5.09 (0.91)
Median	-5.11	-5.05
25th, 75th Percentile	-5.60, -4.39	-5.78, -4.50
Min, Max	-7.9, -3.8	-6.8, -3.0
Week 52		
n	26	27
Mean (SD)	-5.12 (0.89)	-4.82 (0.86)
Median	-5.06	-4.91
25th, 75th Percentile	-5.56, -4.48	-5.59, -4.35
Min, Max	-7.6, -3.8	-6.1, -2.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	26	27
Mean (SD)	0.01 (0.28)	0.27 (0.33)
Median	0.06	0.31
25th, 75th Percentile	-0.09, 0.21	-0.03, 0.57
Min, Max	-0.8, 0.4	-0.2, 0.9
LS mean change from baseline (95% CI)	0.06 (-0.10, 0.23)	0.32 (0.19, 0.46)
Difference in LS mean change from baseline (95% CI) ^a		0.26 (0.09, 0.44)
P-value ^b		0.0044

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.89 (0.28, 1.50)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Europe		
Baseline		
n	18	18
Mean (SD)	-5.11 (1.30)	-5.02 (1.47)
Median	-5.34	-5.21
25th, 75th Percentile	-5.82, -4.27	-6.04, -4.29
Min, Max	-7.3, -2.7	-7.7, -1.1
Week 52		
n	18	18
Mean (SD)	-5.21 (1.30)	-4.80 (1.48)
Median	-5.29	-4.79
25th, 75th Percentile	-5.83, -4.51	-5.70, -4.15
Min, Max	-7.5, -2.8	-7.5, -1.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	18
Mean (SD)	-0.10 (0.24)	0.22 (0.22)
Median	-0.12	0.14
25th, 75th Percentile	-0.23, 0.16	0.04, 0.45
Min, Max	-0.6, 0.2	-0.1, 0.6
LS mean change from baseline (95% CI)	-0.09 (-0.22, 0.04)	0.22 (0.08, 0.37)
Difference in LS mean change from baseline (95% CI) ^a		0.31 (0.17, 0.46)
P-value ^b		0.0001

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.51 (0.73, 2.28)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	3
Mean (SD)	-5.20 (1.00)	-6.26 (0.14)
Median	-5.52	-6.33
25th, 75th Percentile	-5.94, -4.46	-6.35, -6.09
Min, Max	-6.0, -3.8	-6.3, -6.1
Week 52		
n	4	3
Mean (SD)	-5.36 (1.19)	-5.87 (0.55)
Median	-5.60	-5.58
25th, 75th Percentile	-6.30, -4.42	-6.51, -5.51
Min, Max	-6.4, -3.8	-6.5, -5.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	4	3
Mean (SD)	-0.15 (0.26)	0.39 (0.52)
Median	-0.11	0.51
25th, 75th Percentile	-0.36, 0.05	-0.18, 0.83
Min, Max	-0.5, 0.1	-0.2, 0.8
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
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NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Rest of World		
Baseline		
n	13	12
Mean (SD)	-5.18 (1.04)	-5.10 (1.00)
Median	-4.81	-4.92
25th, 75th Percentile	-5.78, -4.49	-6.18, -4.22
Min, Max	-7.1, -3.6	-6.4, -3.8
Week 52		
n	13	12
Mean (SD)	-5.04 (1.22)	-4.92 (0.92)
Median	-4.81	-4.72
25th, 75th Percentile	-5.30, -4.27	-5.62, -4.12
Min, Max	-7.8, -3.4	-6.6, -3.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	0.13 (0.30)	0.19 (0.39)
Median	0.16	0.11
25th, 75th Percentile	0.06, 0.34	-0.07, 0.44
Min, Max	-0.7, 0.5	-0.4, 1.0
LS mean change from baseline (95% CI)	0.01 (-0.20, 0.23)	0.18 (-0.05, 0.40)
Difference in LS mean change from baseline (95% CI) ^a		0.16 (-0.12, 0.45)
P-value ^b		0.2374

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.2.14.1
Analysis of Covariance of Height Z-Score at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Height Z-Score	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.54 (-0.35, 1.40)
P-value for interaction term,treatment * [Region]		0.2882

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing standing height at Week 52 (i.e. Day 365) will be imputed by applying the baseline AGV (cm/yr) to the last available height assessment. Based on this imputed standing height, the height z-score and AGV at Week 52 will be calculated.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.02.014.001.000_mod_sub_reg_haz_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.7.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	31
Mean (SD)	1.97 (0.21)	1.96 (0.20)
Median	1.98	1.99
25th, 75th Percentile	1.83, 2.05	1.88, 2.09
Min, Max	1.5, 2.6	1.3, 2.2
Week 52		
n	33	31
Mean (SD)	1.92 (0.17)	1.94 (0.22)
Median	1.92	1.93
25th, 75th Percentile	1.83, 2.03	1.86, 2.04
Min, Max	1.6, 2.3	1.3, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Missing sitting height and standing height at Week 52 will be imputed by applying the baseline AGV for sitting height and standing height to the last available sitting/standing height assessment. Based on this imputed sitting height and standing height at Week 52, the upper:lower body segment ratio will be calculated. See SAP for additional imputation details.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.03.007.001.000_mod_sub_sex_bodrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.7.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	33	31
Mean (SD)	-0.05 (0.09)	-0.02 (0.14)
Median	-0.03	-0.03
25th, 75th Percentile	-0.09, 0.02	-0.10, 0.01
Min, Max	-0.4, 0.0	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.05 (-0.10, 0.01)	-0.01 (-0.07, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.03 (-0.03, 0.09)
P-value ^b		0.2555

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

Missing sitting height and standing height at Week 52 will be imputed by applying the baseline AGV for sitting height and standing height to the last available sitting/standing height assessment. Based on this imputed sitting height and standing height at Week 52, the upper:lower body segment ratio will be calculated. See SAP for additional imputation details.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.03.007.001.000_mod_sub_sex_bodrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.7.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.29 (-0.21, 0.79)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.7.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	29
Mean (SD)	2.06 (0.20)	2.01 (0.18)
Median	2.05	2.05
25th, 75th Percentile	1.91, 2.16	1.89, 2.16
Min, Max	1.6, 2.4	1.5, 2.3
Week 52		
n	28	29
Mean (SD)	2.05 (0.17)	1.97 (0.19)
Median	2.05	1.99
25th, 75th Percentile	1.90, 2.19	1.87, 2.07
Min, Max	1.7, 2.4	1.5, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

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Table 14.2.3.7.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	29
Mean (SD)	-0.01 (0.07)	-0.04 (0.07)
Median	0.01	-0.04
25th, 75th Percentile	-0.04, 0.04	-0.08, -0.01
Min, Max	-0.2, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.01 (-0.04, 0.02)	-0.04 (-0.06, -0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.07, 0.01)
P-value ^b		0.1905

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and sex interaction.

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Table 14.2.3.7.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Sex Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.37 (-0.91, 0.18)
P-value for interaction term, treatment * [Sex]		0.0915

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Baseline		
n	24	31
Mean (SD)	2.14 (0.21)	2.04 (0.15)
Median	2.14	2.09
25th, 75th Percentile	2.01, 2.30	1.89, 2.16
Min, Max	1.6, 2.6	1.8, 2.3
Week 52		
n	24	31
Mean (SD)	2.09 (0.16)	2.02 (0.16)
Median	2.08	2.03
25th, 75th Percentile	1.97, 2.22	1.87, 2.15
Min, Max	1.8, 2.4	1.8, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and age interaction.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	31
Mean (SD)	-0.05 (0.11)	-0.02 (0.13)
Median	-0.01	-0.04
25th, 75th Percentile	-0.09, 0.02	-0.10, 0.01
Min, Max	-0.4, 0.1	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.04 (-0.10, 0.01)	-0.02 (-0.07, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.05, 0.09)
P-value ^b		0.5478

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.17 (-0.38, 0.71)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	17
Mean (SD)	1.93 (0.18)	1.99 (0.13)
Median	1.92	2.01
25th, 75th Percentile	1.83, 2.05	1.93, 2.05
Min, Max	1.5, 2.4	1.6, 2.2
Week 52		
n	24	17
Mean (SD)	1.91 (0.15)	1.96 (0.15)
Median	1.90	1.96
25th, 75th Percentile	1.83, 2.02	1.89, 2.01
Min, Max	1.6, 2.2	1.6, 2.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	17
Mean (SD)	-0.02 (0.07)	-0.04 (0.09)
Median	0.01	-0.03
25th, 75th Percentile	-0.06, 0.03	-0.10, 0.05
Min, Max	-0.2, 0.0	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.01 (-0.07, 0.05)	-0.04 (-0.09, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.08, 0.03)
P-value ^b		0.3603

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.33 (-1.02, 0.37)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	12
Mean (SD)	1.92 (0.13)	1.82 (0.28)
Median	1.93	1.87
25th, 75th Percentile	1.82, 1.99	1.64, 2.03
Min, Max	1.7, 2.1	1.3, 2.2
Week 52		
n	13	12
Mean (SD)	1.90 (0.15)	1.79 (0.28)
Median	1.90	1.84
25th, 75th Percentile	1.79, 2.00	1.61, 1.99
Min, Max	1.7, 2.2	1.3, 2.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	-0.02 (0.06)	-0.03 (0.05)
Median	-0.01	-0.04
25th, 75th Percentile	-0.05, 0.01	-0.06, 0.00
Min, Max	-0.1, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	-0.01 (-0.04, 0.02)	-0.05 (-0.09, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.09, 0.02)
P-value ^b		0.2133

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.8.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.67 (-1.70, 0.38)
P-value for interaction term, treatment * [Age at Baseline]		0.6506

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.9.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	48
Mean (SD)	2.03 (0.21)	2.00 (0.17)
Median	2.02	2.01
25th, 75th Percentile	1.91, 2.14	1.90, 2.12
Min, Max	1.5, 2.6	1.3, 2.3
Week 52		
n	48	48
Mean (SD)	1.99 (0.18)	1.98 (0.18)
Median	1.99	1.97
25th, 75th Percentile	1.87, 2.10	1.87, 2.07
Min, Max	1.6, 2.4	1.3, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.9.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	48	48
Mean (SD)	-0.03 (0.09)	-0.03 (0.12)
Median	-0.02	-0.04
25th, 75th Percentile	-0.07, 0.02	-0.10, 0.01
Min, Max	-0.4, 0.1	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.03 (-0.06, 0.00)	-0.03 (-0.06, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.04, 0.05)
P-value ^b		0.8477

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

Missing sitting height and standing height at Week 52 will be imputed by applying the baseline AGV for sitting height and standing height to the last available sitting/standing height assessment. Based on this imputed sitting height and standing height at Week 52, the upper:lower body segment ratio will be calculated. See SAP for additional imputation details.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.9.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.04 (-0.37, 0.45)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.9.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	12
Mean (SD)	1.96 (0.19)	1.89 (0.27)
Median	1.93	1.94
25th, 75th Percentile	1.88, 1.99	1.72, 2.13
Min, Max	1.7, 2.4	1.4, 2.2
Week 52		
n	13	12
Mean (SD)	1.94 (0.17)	1.87 (0.27)
Median	1.94	1.93
25th, 75th Percentile	1.86, 2.00	1.71, 2.08
Min, Max	1.7, 2.2	1.4, 2.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and tanner stage interaction.

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Table 14.2.3.9.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	-0.02 (0.08)	-0.02 (0.06)
Median	0.00	-0.03
25th, 75th Percentile	-0.05, 0.02	-0.05, 0.02
Min, Max	-0.2, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.02 (-0.07, 0.03)	-0.04 (-0.10, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.11, 0.06)
P-value ^b		0.5560

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.9.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.33 (-1.41, 0.76)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.9934

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	28
Mean (SD)	1.99 (0.22)	1.97 (0.18)
Median	1.99	1.99
25th, 75th Percentile	1.86, 2.07	1.90, 2.08
Min, Max	1.5, 2.6	1.3, 2.2
Week 52		
n	28	28
Mean (SD)	1.94 (0.17)	1.96 (0.19)
Median	1.94	1.94
25th, 75th Percentile	1.83, 2.05	1.88, 2.04
Min, Max	1.6, 2.3	1.3, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	28
Mean (SD)	-0.05 (0.10)	-0.02 (0.14)
Median	-0.03	-0.03
25th, 75th Percentile	-0.10, 0.02	-0.11, 0.02
Min, Max	-0.4, 0.0	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.05 (-0.10, -0.01)	-0.02 (-0.07, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		0.03 (-0.03, 0.10)
P-value ^b		0.3037

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Upper to Lower Body Segment Ratio		
SMD (95% CI) ^c		0.28 (-0.25, 0.81)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	2.08 (0.19)	2.05 (0.14)
Median	2.08	2.07
25th, 75th Percentile	1.94, 2.17	1.90, 2.16
Min, Max	1.6, 2.4	1.8, 2.3
Week 52		
n	20	20
Mean (SD)	2.07 (0.17)	2.00 (0.16)
Median	2.08	1.98
25th, 75th Percentile	1.90, 2.19	1.87, 2.11
Min, Max	1.8, 2.4	1.8, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	20
Mean (SD)	-0.01 (0.06)	-0.04 (0.07)
Median	0.00	-0.06
25th, 75th Percentile	-0.04, 0.03	-0.09, -0.01
Min, Max	-0.2, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.04, 0.03)	-0.05 (-0.08, -0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.04 (-0.09, 0.01)
P-value ^b		0.0874

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Upper to Lower Body Segment Ratio		
SMD (95% CI) ^c		-0.60 (-1.28, 0.09)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	1.87 (0.14)	1.78 (0.37)
Median	1.93	1.81
25th, 75th Percentile	1.80, 1.98	1.40, 2.14
Min, Max	1.7, 2.0	1.4, 2.1
Week 52		
n	5	3
Mean (SD)	1.84 (0.13)	1.75 (0.37)
Median	1.86	1.81
25th, 75th Percentile	1.75, 1.92	1.36, 2.09
Min, Max	1.7, 2.0	1.4, 2.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	5	3
Mean (SD)	-0.04 (0.05)	-0.03 (0.02)
Median	-0.01	-0.04
25th, 75th Percentile	-0.05, 0.00	-0.05, 0.00
Min, Max	-0.1, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	-0.01 (-0.10, 0.09)	-0.08 (-0.22, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.29, 0.15)
P-value ^b		0.3796

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Upper to Lower Body Segment Ratio		
SMD (95% CI) ^c		-1.68 (-5.02, 1.88)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	9
Mean (SD)	2.01 (0.21)	1.93 (0.25)
Median	1.92	2.02
25th, 75th Percentile	1.89, 2.09	1.81, 2.13
Min, Max	1.8, 2.4	1.5, 2.2
Week 52		
n	8	9
Mean (SD)	2.00 (0.16)	1.91 (0.25)
Median	1.95	2.00
25th, 75th Percentile	1.92, 2.13	1.80, 2.07
Min, Max	1.7, 2.2	1.5, 2.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

Missing sitting height and standing height at Week 52 will be imputed by applying the baseline AGV for sitting height and standing height to the last available sitting/standing height assessment. Based on this imputed sitting height and standing height at Week 52, the upper:lower body segment ratio will be calculated. See SAP for additional imputation details.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	9
Mean (SD)	-0.01 (0.09)	-0.02 (0.07)
Median	0.02	-0.02
25th, 75th Percentile	-0.03, 0.04	-0.05, 0.05
Min, Max	-0.2, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.01 (-0.08, 0.07)	-0.03 (-0.10, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.14, 0.09)
P-value ^b		0.6976

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and stratum interaction.

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Table 14.2.3.10.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Strata Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.23 (-1.39, 0.93)
P-value for interaction term, treatment * [Strata]		0.3512

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	15
Mean (SD)	2.12 (0.19)	2.08 (0.12)
Median	2.12	2.10
25th, 75th Percentile	2.02, 2.17	2.04, 2.16
Min, Max	1.7, 2.4	1.8, 2.3
Week 52		
n	10	15
Mean (SD)	2.12 (0.17)	2.04 (0.11)
Median	2.13	2.06
25th, 75th Percentile	2.03, 2.19	1.94, 2.15
Min, Max	1.8, 2.4	1.9, 2.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	10	15
Mean (SD)	-0.01 (0.06)	-0.04 (0.07)
Median	0.02	-0.04
25th, 75th Percentile	-0.04, 0.03	-0.10, 0.04
Min, Max	-0.2, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.03 (-0.02, 0.09)	-0.03 (-0.07, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.13, -0.01)
P-value ^b		0.0275

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-1.11 (-2.07, -0.12)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	2.02 (0.22)	2.04 (0.13)
Median	1.98	2.01
25th, 75th Percentile	1.88, 2.13	1.91, 2.17
Min, Max	1.6, 2.6	1.9, 2.2
Week 52		
n	24	18
Mean (SD)	1.99 (0.17)	2.01 (0.15)
Median	1.96	2.00
25th, 75th Percentile	1.86, 2.09	1.89, 2.11
Min, Max	1.7, 2.3	1.8, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	18
Mean (SD)	-0.02 (0.08)	-0.03 (0.08)
Median	0.01	-0.02
25th, 75th Percentile	-0.07, 0.02	-0.10, 0.01
Min, Max	-0.2, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.03 (-0.08, 0.01)	-0.03 (-0.08, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.06, 0.05)
P-value ^b		0.9490

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
 Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.02 (-0.67, 0.63)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	2.02 (0.17)	1.97 (0.13)
Median	2.00	1.99
25th, 75th Percentile	1.90, 2.07	1.86, 2.07
Min, Max	1.8, 2.4	1.8, 2.2
Week 52		
n	19	22
Mean (SD)	1.97 (0.13)	1.95 (0.16)
Median	1.94	1.94
25th, 75th Percentile	1.88, 2.09	1.81, 2.04
Min, Max	1.7, 2.2	1.8, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	22
Mean (SD)	-0.05 (0.11)	-0.02 (0.16)
Median	-0.02	-0.05
25th, 75th Percentile	-0.06, 0.03	-0.10, 0.03
Min, Max	-0.4, 0.0	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.02 (-0.12, 0.08)	0.00 (-0.08, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.08, 0.12)
P-value ^b		0.7122

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.13 (-0.54, 0.79)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	1.85 (0.18)	1.55 (0.26)
Median	1.90	1.47
25th, 75th Percentile	1.70, 2.01	1.40, 1.64
Min, Max	1.5, 2.0	1.3, 2.0
Week 52		
n	8	5
Mean (SD)	1.80 (0.17)	1.52 (0.23)
Median	1.85	1.46
25th, 75th Percentile	1.63, 1.94	1.36, 1.62
Min, Max	1.6, 2.0	1.3, 1.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	5
Mean (SD)	-0.05 (0.06)	-0.03 (0.04)
Median	-0.06	-0.02
25th, 75th Percentile	-0.10, 0.01	-0.04, -0.02
Min, Max	-0.1, 0.0	-0.1, 0.0
LS mean change from baseline (95% CI)	0.03 (-0.02, 0.09)	-0.07 (-0.11, -0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.10 (-0.17, -0.02)
P-value ^b		0.0184

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.03.011.001.000_mod_sub_bhgt_bodrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.11.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-3.61 (-6.53, -0.54)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.4133

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and height z-score interaction.

Missing sitting height and standing height at Week 52 will be imputed by applying the baseline AGV for sitting height and standing height to the last available sitting/standing height assessment. Based on this imputed sitting height and standing height at Week 52, the upper:lower body segment ratio will be calculated. See SAP for additional imputation details.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	19
Mean (SD)	2.03 (0.22)	1.97 (0.23)
Median	2.02	1.97
25th, 75th Percentile	1.83, 2.12	1.87, 2.15
Min, Max	1.7, 2.6	1.3, 2.3
Week 52		
n	19	19
Mean (SD)	1.98 (0.18)	1.99 (0.24)
Median	1.95	2.01
25th, 75th Percentile	1.85, 2.07	1.87, 2.18
Min, Max	1.6, 2.3	1.3, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	19
Mean (SD)	-0.05 (0.09)	0.02 (0.16)
Median	-0.04	0.01
25th, 75th Percentile	-0.12, 0.02	-0.08, 0.07
Min, Max	-0.2, 0.1	-0.2, 0.6
LS mean change from baseline (95% CI)	-0.06 (-0.14, 0.03)	0.02 (-0.09, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		0.07 (-0.03, 0.17)
P-value ^b		0.1770

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and AGV interaction.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.50 (-0.23, 1.23)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	1.97 (0.18)	1.98 (0.14)
Median	1.93	2.01
25th, 75th Percentile	1.88, 2.07	1.88, 2.05
Min, Max	1.7, 2.4	1.6, 2.2
Week 52		
n	18	14
Mean (SD)	1.96 (0.17)	1.90 (0.13)
Median	1.93	1.93
25th, 75th Percentile	1.83, 2.03	1.82, 1.98
Min, Max	1.7, 2.3	1.6, 2.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	14
Mean (SD)	-0.02 (0.06)	-0.07 (0.04)
Median	0.00	-0.07
25th, 75th Percentile	-0.06, 0.03	-0.10, -0.03
Min, Max	-0.2, 0.0	-0.1, 0.0
LS mean change from baseline (95% CI)	-0.01 (-0.04, 0.02)	-0.07 (-0.10, -0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.09, -0.01)
P-value ^b		0.0182

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-1.00 (-1.81, -0.17)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	27
Mean (SD)	2.03 (0.22)	1.99 (0.20)
Median	2.00	2.05
25th, 75th Percentile	1.92, 2.14	1.89, 2.14
Min, Max	1.5, 2.4	1.4, 2.2
Week 52		
n	24	27
Mean (SD)	2.00 (0.19)	1.96 (0.21)
Median	1.99	1.99
25th, 75th Percentile	1.88, 2.11	1.87, 2.09
Min, Max	1.6, 2.4	1.4, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	27
Mean (SD)	-0.03 (0.10)	-0.04 (0.07)
Median	0.00	-0.04
25th, 75th Percentile	-0.06, 0.03	-0.08, 0.00
Min, Max	-0.4, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.02 (-0.08, 0.04)	-0.03 (-0.07, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.08, 0.04)
P-value ^b		0.5907

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.12.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.18 (-0.83, 0.47)
P-value for interaction term, treatment * [Baseline AGV]		0.0221

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.13.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	45
Mean (SD)	2.03 (0.21)	1.99 (0.21)
Median	2.02	2.02
25th, 75th Percentile	1.91, 2.12	1.89, 2.15
Min, Max	1.5, 2.6	1.3, 2.3
Week 52		
n	41	45
Mean (SD)	2.00 (0.17)	1.96 (0.21)
Median	2.00	1.98
25th, 75th Percentile	1.88, 2.10	1.86, 2.07
Min, Max	1.6, 2.4	1.3, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.13.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	41	45
Mean (SD)	-0.03 (0.09)	-0.04 (0.08)
Median	-0.01	-0.04
25th, 75th Percentile	-0.05, 0.02	-0.10, 0.01
Min, Max	-0.4, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.04 (-0.07, 0.00)	-0.04 (-0.07, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.04, 0.04)
P-value ^b		0.9156

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.13.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.02 (-0.46, 0.41)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.13.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	15
Mean (SD)	1.97 (0.21)	1.94 (0.15)
Median	1.95	1.97
25th, 75th Percentile	1.84, 2.04	1.81, 2.05
Min, Max	1.7, 2.4	1.6, 2.2
Week 52		
n	20	15
Mean (SD)	1.95 (0.20)	1.95 (0.18)
Median	1.92	1.93
25th, 75th Percentile	1.80, 2.06	1.87, 2.09
Min, Max	1.6, 2.3	1.6, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and ethnicity interaction.

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Table 14.2.3.13.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	15
Mean (SD)	-0.03 (0.07)	0.01 (0.16)
Median	0.00	-0.03
25th, 75th Percentile	-0.07, 0.03	-0.07, 0.04
Min, Max	-0.2, 0.0	-0.1, 0.6
LS mean change from baseline (95% CI)	-0.03 (-0.11, 0.05)	0.02 (-0.06, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (-0.05, 0.14)
P-value ^b		0.3035

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.13.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.38 (-0.34, 1.10)
P-value for interaction term,treatment * [Ethnicity]		0.2556

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
North America		
Baseline		
n	26	27
Mean (SD)	1.95 (0.15)	1.96 (0.18)
Median	1.98	1.97
25th, 75th Percentile	1.88, 2.05	1.81, 2.14
Min, Max	1.6, 2.3	1.5, 2.2
Week 52		
n	26	27
Mean (SD)	1.93 (0.14)	1.95 (0.20)
Median	1.91	1.91
25th, 75th Percentile	1.83, 2.03	1.84, 2.09
Min, Max	1.6, 2.2	1.5, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	26	27
Mean (SD)	-0.03 (0.10)	-0.01 (0.14)
Median	0.01	-0.02
25th, 75th Percentile	-0.06, 0.02	-0.10, 0.03
Min, Max	-0.4, 0.1	-0.2, 0.6
LS mean change from baseline (95% CI)	0.00 (-0.07, 0.07)	0.00 (-0.05, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.08, 0.07)
P-value ^b		0.9963

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.00 (-0.59, 0.58)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Europe		
Baseline		
n	18	18
Mean (SD)	2.08 (0.23)	1.98 (0.26)
Median	2.06	2.06
25th, 75th Percentile	1.94, 2.16	1.89, 2.16
Min, Max	1.5, 2.6	1.3, 2.3
Week 52		
n	18	18
Mean (SD)	2.04 (0.19)	1.93 (0.26)
Median	2.02	2.00
25th, 75th Percentile	1.93, 2.19	1.82, 2.07
Min, Max	1.6, 2.4	1.3, 2.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	18
Mean (SD)	-0.04 (0.08)	-0.05 (0.08)
Median	-0.01	-0.04
25th, 75th Percentile	-0.09, 0.03	-0.12, 0.03
Min, Max	-0.2, 0.0	-0.2, 0.1
LS mean change from baseline (95% CI)	-0.06 (-0.11, 0.00)	-0.07 (-0.13, -0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.08, 0.04)
P-value ^b		0.5701

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.20 (-0.87, 0.48)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	3
Mean (SD)	2.18 (0.35)	2.14 (0.05)
Median	2.30	2.13
25th, 75th Percentile	1.97, 2.39	2.10, 2.20
Min, Max	1.7, 2.4	2.1, 2.2
Week 52		
n	4	3
Mean (SD)	2.13 (0.30)	2.11 (0.10)
Median	2.26	2.15
25th, 75th Percentile	1.96, 2.29	1.99, 2.17
Min, Max	1.7, 2.3	2.0, 2.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	4	3
Mean (SD)	-0.05 (0.09)	-0.03 (0.08)
Median	-0.04	-0.04
25th, 75th Percentile	-0.12, 0.02	-0.11, 0.05
Min, Max	-0.2, 0.0	-0.1, 0.0
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Upper to Lower Body Segment Ratio		

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Rest of World		
Baseline		
n	13	12
Mean (SD)	1.98 (0.18)	2.01 (0.10)
Median	1.96	2.02
25th, 75th Percentile	1.83, 2.07	1.92, 2.07
Min, Max	1.8, 2.4	1.9, 2.2
Week 52		
n	13	12
Mean (SD)	1.95 (0.15)	1.96 (0.11)
Median	1.94	1.98
25th, 75th Percentile	1.84, 2.07	1.88, 2.04
Min, Max	1.7, 2.3	1.8, 2.1

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	-0.03 (0.06)	-0.04 (0.04)
Median	-0.03	-0.05
25th, 75th Percentile	-0.05, 0.03	-0.07, -0.03
Min, Max	-0.2, 0.0	-0.1, 0.1
LS mean change from baseline (95% CI)	-0.03 (-0.06, 0.00)	-0.06 (-0.09, -0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.07, 0.02)
P-value ^b		0.1887

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

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Table 14.2.3.14.1
Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Region Upper to Lower Body Segment Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.60 (-1.47, 0.29)
P-value for interaction term,treatment * [Region]		0.8961

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV and baseline height z-score. For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, and treatment and region interaction.

Missing sitting height and standing height at Week 52 will be imputed by applying the baseline AGV for sitting height and standing height to the last available sitting/standing height assessment. Based on this imputed sitting height and standing height at Week 52, the upper:lower body segment ratio will be calculated. See SAP for additional imputation details.

Report: mi897809 19JUN2023 07:47 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.03.014.001.000_mod_sub_reg_bodrt_301_fas.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge_sub_301.sas, Database: N/A

Table 14.2.10.1.1
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Annualized Growth Velocity (AGV) at Week 52 for BMN111-301
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.2562
Baseline Age Group*Treatment Interaction	0.0310
Baseline Tanner Stage*Treatment Interaction	0.0419
Strata*Treatment Interaction	0.2055
Baseline Height Z-Score Category*Treatment Interaction	0.3158
Baseline AGV Category*Treatment Interaction	0.8219
Ethnicity*Treatment Interaction	0.8672
Region*Treatment Interaction	0.7439

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 19JUN2023 07:44 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.001.001.000_mod_agv_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.2.1
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Height Z-Score at Week 52 for BMN111-301
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.1434
Baseline Age Group*Treatment Interaction	0.2675
Baseline Tanner Stage*Treatment Interaction	0.7461
Strata*Treatment Interaction	0.3315
Baseline Height Z-Score Category*Treatment Interaction	0.4818
Baseline AGV Category*Treatment Interaction	0.2511
Ethnicity*Treatment Interaction	0.4489
Region*Treatment Interaction	0.2882

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 19JUN2023 07:44 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.002.001.000_mod_haz_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.3.1
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper to Lower Body Segment Ratio at Week 52 for BMN111-301
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.0915
Baseline Age Group*Treatment Interaction	0.6506
Baseline Tanner Stage*Treatment Interaction	0.9934
Strata*Treatment Interaction	0.3512
Baseline Height Z-Score Category*Treatment Interaction	0.4133
Baseline AGV Category*Treatment Interaction	0.0221
Ethnicity*Treatment Interaction	0.2556
Region*Treatment Interaction	0.8961

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 19JUN2023 07:44 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.003.001.000_mod_bodrt_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval_sub_301.sas, Database: N/A

Table 14.2.4.7.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	30
Mean (SD)	1.06 (0.08)	1.11 (0.16)
Median	1.06	1.08
25th, 75th Percentile	1.01, 1.10	1.02, 1.15
Min, Max	0.9, 1.3	0.8, 1.6
Week 52		
n	33	30
Mean (SD)	1.07 (0.08)	1.10 (0.14)
Median	1.07	1.05
25th, 75th Percentile	1.02, 1.13	1.02, 1.15
Min, Max	0.9, 1.3	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.007.001.000_mod_sub_sex_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.7.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	33	30
Mean (SD)	0.01 (0.10)	0.00 (0.09)
Median	-0.01	-0.01
25th, 75th Percentile	-0.05, 0.09	-0.05, 0.05
Min, Max	-0.2, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	-0.01 (-0.06, 0.04)	-0.02 (-0.07, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.07, 0.04)
P-value ^b		0.5475

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.007.001.000_mod_sub_sex_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.7.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Sex for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.15 (-0.65, 0.35)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.007.001.000_mod_sub_sex_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.7.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	28
Mean (SD)	1.04 (0.08)	1.05 (0.10)
Median	1.04	1.06
25th, 75th Percentile	0.97, 1.10	0.99, 1.09
Min, Max	0.9, 1.2	0.8, 1.3
Week 52		
n	28	28
Mean (SD)	1.11 (0.10)	1.08 (0.12)
Median	1.10	1.06
25th, 75th Percentile	1.03, 1.19	0.99, 1.15
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.007.001.000_mod_sub_sex_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.7.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	28
Mean (SD)	0.07 (0.10)	0.02 (0.11)
Median	0.04	0.00
25th, 75th Percentile	0.01, 0.12	-0.04, 0.10
Min, Max	-0.1, 0.3	-0.1, 0.3
LS mean change from baseline (95% CI)	0.08 (0.03, 0.12)	0.04 (0.00, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.04 (-0.10, 0.02)
P-value ^b		0.2028

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.007.001.000_mod_sub_sex_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.7.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Sex for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.36 (-0.91, 0.19)
P-value for interaction term, treatment * [Sex]		0.5226

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.007.001.000_mod_sub_sex_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Baseline		
n	24	31
Mean (SD)	1.03 (0.09)	1.07 (0.11)
Median	1.03	1.06
25th, 75th Percentile	0.96, 1.09	1.02, 1.12
Min, Max	0.9, 1.3	0.8, 1.3
Week 52		
n	24	31
Mean (SD)	1.07 (0.10)	1.06 (0.09)
Median	1.06	1.05
25th, 75th Percentile	0.99, 1.16	0.99, 1.09
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	31
Mean (SD)	0.04 (0.13)	-0.01 (0.09)
Median	0.02	-0.02
25th, 75th Percentile	-0.04, 0.11	-0.06, 0.04
Min, Max	-0.2, 0.3	-0.1, 0.2
LS mean change from baseline (95% CI)	0.04 (0.00, 0.09)	-0.01 (-0.05, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.11, 0.01)
P-value ^b		0.1183

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.44 (-0.99, 0.11)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	16
Mean (SD)	1.06 (0.07)	1.06 (0.09)
Median	1.06	1.06
25th, 75th Percentile	1.01, 1.11	0.98, 1.14
Min, Max	0.9, 1.2	0.9, 1.2
Week 52		
n	24	16
Mean (SD)	1.11 (0.08)	1.07 (0.12)
Median	1.09	1.05
25th, 75th Percentile	1.03, 1.17	0.98, 1.14
Min, Max	1.0, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	16
Mean (SD)	0.04 (0.09)	0.01 (0.09)
Median	0.04	0.02
25th, 75th Percentile	-0.02, 0.11	-0.04, 0.07
Min, Max	-0.1, 0.3	-0.2, 0.1
LS mean change from baseline (95% CI)	0.03 (-0.03, 0.10)	0.02 (-0.04, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.08, 0.05)
P-value ^b		0.6032

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.19 (-0.88, 0.51)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	11
Mean (SD)	1.08 (0.07)	1.15 (0.22)
Median	1.09	1.05
25th, 75th Percentile	1.03, 1.12	1.00, 1.32
Min, Max	1.0, 1.2	0.9, 1.6
Week 52		
n	13	11
Mean (SD)	1.09 (0.08)	1.21 (0.18)
Median	1.10	1.20
25th, 75th Percentile	1.04, 1.14	1.07, 1.32
Min, Max	0.9, 1.2	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.01 (0.05)	0.06 (0.14)
Median	0.01	0.02
25th, 75th Percentile	-0.03, 0.04	-0.06, 0.17
Min, Max	-0.1, 0.1	-0.1, 0.3
LS mean change from baseline (95% CI)	0.00 (-0.06, 0.06)	0.07 (-0.01, 0.14)
Difference in LS mean change from baseline (95% CI) ^a		0.07 (-0.03, 0.17)
P-value ^b		0.1696

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.8.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.77 (-0.32, 1.84)
P-value for interaction term, treatment * [Age at Baseline]		0.2776

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.008.001.000_mod_sub_age_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.9.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	47
Mean (SD)	1.05 (0.08)	1.07 (0.12)
Median	1.06	1.06
25th, 75th Percentile	0.99, 1.10	1.02, 1.12
Min, Max	0.9, 1.3	0.8, 1.5
Week 52		
n	48	47
Mean (SD)	1.08 (0.09)	1.07 (0.12)
Median	1.07	1.05
25th, 75th Percentile	1.02, 1.16	0.99, 1.10
Min, Max	0.9, 1.3	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.009.001.000_mod_sub_tan_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.9.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	48	47
Mean (SD)	0.03 (0.11)	0.00 (0.09)
Median	0.01	-0.01
25th, 75th Percentile	-0.04, 0.11	-0.05, 0.05
Min, Max	-0.2, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	0.03 (0.00, 0.06)	0.00 (-0.03, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.07, 0.01)
P-value ^b		0.1602

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.9.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.30 (-0.71, 0.12)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.009.001.000_mod_sub_tan_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.9.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	11
Mean (SD)	1.06 (0.07)	1.12 (0.19)
Median	1.06	1.05
25th, 75th Percentile	1.02, 1.11	0.99, 1.21
Min, Max	1.0, 1.2	0.9, 1.6
Week 52		
n	13	11
Mean (SD)	1.11 (0.08)	1.17 (0.16)
Median	1.11	1.17
25th, 75th Percentile	1.04, 1.18	1.06, 1.31
Min, Max	0.9, 1.2	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.9.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.05 (0.09)	0.05 (0.14)
Median	0.04	0.01
25th, 75th Percentile	-0.01, 0.08	-0.06, 0.15
Min, Max	-0.1, 0.3	-0.1, 0.3
LS mean change from baseline (95% CI)	0.03 (-0.04, 0.10)	0.03 (-0.06, 0.11)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.12, 0.12)
P-value ^b		0.9916

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.9.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.01 (-1.07, 1.09)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.7417

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.009.001.000_mod_sub_tan_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	27
Mean (SD)	1.06 (0.08)	1.08 (0.13)
Median	1.06	1.05
25th, 75th Percentile	1.00, 1.10	1.02, 1.13
Min, Max	0.9, 1.3	0.8, 1.5
Week 52		
n	28	27
Mean (SD)	1.07 (0.08)	1.08 (0.13)
Median	1.07	1.05
25th, 75th Percentile	1.01, 1.13	1.02, 1.14
Min, Max	0.9, 1.3	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	27
Mean (SD)	0.01 (0.11)	0.01 (0.09)
Median	-0.01	0.00
25th, 75th Percentile	-0.05, 0.10	-0.05, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.05)	0.01 (-0.03, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.06, 0.05)
P-value ^b		0.8342

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.010.001.000_mod_sub_strata_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.06 (-0.59, 0.47)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.010.001.000_mod_sub_strata_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	1.04 (0.09)	1.06 (0.10)
Median	1.04	1.07
25th, 75th Percentile	0.98, 1.10	1.02, 1.10
Min, Max	0.9, 1.2	0.8, 1.3
Week 52		
n	20	20
Mean (SD)	1.10 (0.10)	1.06 (0.10)
Median	1.10	1.05
25th, 75th Percentile	1.02, 1.19	0.99, 1.10
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	20
Mean (SD)	0.06 (0.10)	0.00 (0.09)
Median	0.03	-0.02
25th, 75th Percentile	0.00, 0.13	-0.07, 0.03
Min, Max	-0.1, 0.3	-0.1, 0.2
LS mean change from baseline (95% CI)	0.06 (0.01, 0.11)	-0.01 (-0.05, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.07 (-0.14, 0.00)
P-value ^b		0.0601

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.66 (-1.34, 0.03)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.010.001.000_mod_sub_strata_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	1.09 (0.06)	1.36 (0.18)
Median	1.09	1.32
25th, 75th Percentile	1.06, 1.12	1.20, 1.56
Min, Max	1.0, 1.2	1.2, 1.6
Week 52		
n	5	3
Mean (SD)	1.09 (0.07)	1.28 (0.14)
Median	1.11	1.27
25th, 75th Percentile	1.04, 1.13	1.15, 1.44
Min, Max	1.0, 1.2	1.1, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	5	3
Mean (SD)	0.00 (0.07)	-0.08 (0.04)
Median	-0.01	-0.06
25th, 75th Percentile	-0.04, 0.02	-0.13, -0.05
Min, Max	-0.1, 0.1	-0.1, -0.1
LS mean change from baseline (95% CI)	-0.06 (-0.17, 0.05)	0.02 (-0.15, 0.19)
Difference in LS mean change from baseline (95% CI) ^a		0.08 (-0.18, 0.34)
P-value ^b		0.4178

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.53 (-1.98, 4.84)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	8
Mean (SD)	1.04 (0.07)	1.03 (0.09)
Median	1.04	1.00
25th, 75th Percentile	0.97, 1.09	0.97, 1.06
Min, Max	1.0, 1.1	0.9, 1.2
Week 52		
n	8	8
Mean (SD)	1.12 (0.10)	1.13 (0.15)
Median	1.12	1.13
25th, 75th Percentile	1.06, 1.20	1.01, 1.26
Min, Max	0.9, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.10.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	8
Mean (SD)	0.08 (0.09)	0.10 (0.13)
Median	0.07	0.10
25th, 75th Percentile	0.03, 0.11	0.00, 0.19
Min, Max	0.0, 0.3	-0.1, 0.3
LS mean change from baseline (95% CI)	0.09 (-0.01, 0.19)	0.09 (-0.02, 0.19)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.16, 0.15)
P-value ^b		0.9600

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.10.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.03 (-1.19, 1.13)
P-value for interaction term, treatment * [Strata]		0.3813

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	13
Mean (SD)	1.06 (0.09)	1.07 (0.10)
Median	1.09	1.05
25th, 75th Percentile	0.98, 1.13	1.00, 1.15
Min, Max	0.9, 1.2	0.9, 1.3
Week 52		
n	10	13
Mean (SD)	1.09 (0.09)	1.11 (0.16)
Median	1.08	1.08
25th, 75th Percentile	1.01, 1.18	0.96, 1.25
Min, Max	1.0, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	10	13
Mean (SD)	0.04 (0.09)	0.03 (0.12)
Median	0.01	0.01
25th, 75th Percentile	-0.01, 0.08	-0.03, 0.09
Min, Max	-0.1, 0.2	-0.1, 0.3
LS mean change from baseline (95% CI)	0.06 (-0.03, 0.16)	0.04 (-0.04, 0.13)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.13, 0.09)
P-value ^b		0.7025

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.18 (-1.10, 0.74)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	1.06 (0.08)	1.07 (0.11)
Median	1.06	1.07
25th, 75th Percentile	1.00, 1.10	1.02, 1.13
Min, Max	0.9, 1.3	0.8, 1.3
Week 52		
n	24	18
Mean (SD)	1.09 (0.09)	1.07 (0.10)
Median	1.09	1.04
25th, 75th Percentile	1.02, 1.16	1.02, 1.12
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	18
Mean (SD)	0.03 (0.11)	0.00 (0.09)
Median	0.02	-0.02
25th, 75th Percentile	-0.03, 0.10	-0.06, 0.05
Min, Max	-0.2, 0.2	-0.1, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.02, 0.08)	0.00 (-0.06, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.10, 0.04)
P-value ^b		0.4288

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.27 (-0.92, 0.39)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	1.04 (0.08)	1.06 (0.11)
Median	1.04	1.06
25th, 75th Percentile	0.99, 1.09	1.02, 1.12
Min, Max	0.9, 1.2	0.8, 1.3
Week 52		
n	19	22
Mean (SD)	1.09 (0.08)	1.08 (0.10)
Median	1.08	1.06
25th, 75th Percentile	1.03, 1.16	1.03, 1.14
Min, Max	0.9, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

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Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	22
Mean (SD)	0.05 (0.10)	0.02 (0.11)
Median	0.06	0.01
25th, 75th Percentile	-0.03, 0.12	-0.05, 0.08
Min, Max	-0.1, 0.3	-0.2, 0.2
LS mean change from baseline (95% CI)	0.04 (-0.03, 0.12)	0.02 (-0.04, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.10, 0.05)
P-value ^b		0.5191

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.22 (-0.89, 0.45)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.011.001.000_mod_sub_bhgt_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	1.07 (0.08)	1.23 (0.28)
Median	1.07	1.07
25th, 75th Percentile	0.99, 1.13	1.02, 1.51
Min, Max	1.0, 1.2	1.0, 1.6
Week 52		
n	8	5
Mean (SD)	1.08 (0.11)	1.18 (0.26)
Median	1.05	1.06
25th, 75th Percentile	1.01, 1.17	1.02, 1.44
Min, Max	0.9, 1.3	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	5
Mean (SD)	0.02 (0.12)	-0.05 (0.06)
Median	-0.03	-0.02
25th, 75th Percentile	-0.05, 0.06	-0.10, -0.01
Min, Max	-0.1, 0.3	-0.1, 0.0
LS mean change from baseline (95% CI)	0.09 (-0.12, 0.30)	-0.08 (-0.23, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.17 (-0.46, 0.11)
P-value ^b		0.1817

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.11.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-1.63 (-3.84, 0.71)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.6082

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	18
Mean (SD)	1.07 (0.08)	1.08 (0.13)
Median	1.08	1.05
25th, 75th Percentile	1.01, 1.13	1.01, 1.12
Min, Max	1.0, 1.3	0.9, 1.5
Week 52		
n	19	18
Mean (SD)	1.10 (0.10)	1.07 (0.13)
Median	1.11	1.04
25th, 75th Percentile	1.03, 1.19	1.02, 1.07
Min, Max	0.9, 1.2	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	18
Mean (SD)	0.03 (0.12)	-0.01 (0.06)
Median	0.00	-0.02
25th, 75th Percentile	-0.05, 0.11	-0.05, 0.02
Min, Max	-0.2, 0.2	-0.1, 0.1
LS mean change from baseline (95% CI)	0.06 (0.00, 0.11)	0.02 (-0.04, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.10, 0.03)
P-value ^b		0.3082

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.39 (-1.12, 0.35)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	1.06 (0.07)	1.06 (0.09)
Median	1.07	1.06
25th, 75th Percentile	0.99, 1.10	1.02, 1.09
Min, Max	0.9, 1.2	0.9, 1.3
Week 52		
n	18	14
Mean (SD)	1.09 (0.08)	1.10 (0.12)
Median	1.08	1.06
25th, 75th Percentile	1.02, 1.14	0.99, 1.20
Min, Max	1.0, 1.3	1.0, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	14
Mean (SD)	0.03 (0.08)	0.04 (0.08)
Median	0.01	0.03
25th, 75th Percentile	-0.02, 0.06	-0.04, 0.09
Min, Max	-0.1, 0.3	-0.1, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.05)	0.03 (-0.02, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.04, 0.08)
P-value ^b		0.5964

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.21 (-0.56, 0.98)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	26
Mean (SD)	1.03 (0.08)	1.09 (0.16)
Median	1.04	1.07
25th, 75th Percentile	0.97, 1.08	1.00, 1.20
Min, Max	0.9, 1.2	0.8, 1.6
Week 52		
n	24	26
Mean (SD)	1.08 (0.09)	1.10 (0.14)
Median	1.08	1.07
25th, 75th Percentile	1.02, 1.15	1.02, 1.23
Min, Max	0.9, 1.3	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	26
Mean (SD)	0.05 (0.11)	0.01 (0.13)
Median	0.03	0.00
25th, 75th Percentile	-0.03, 0.11	-0.10, 0.10
Min, Max	-0.1, 0.3	-0.2, 0.3
LS mean change from baseline (95% CI)	0.06 (-0.02, 0.14)	0.00 (-0.06, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.06 (-0.14, 0.02)
P-value ^b		0.1285

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.12.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.52 (-1.18, 0.15)
P-value for interaction term, treatment * [Baseline AGV]		0.5864

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.13.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	44
Mean (SD)	1.06 (0.08)	1.07 (0.14)
Median	1.06	1.05
25th, 75th Percentile	1.00, 1.11	1.00, 1.12
Min, Max	0.9, 1.3	0.8, 1.6
Week 52		
n	41	44
Mean (SD)	1.09 (0.09)	1.10 (0.14)
Median	1.07	1.05
25th, 75th Percentile	1.02, 1.16	1.00, 1.16
Min, Max	0.9, 1.3	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.13.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	41	44
Mean (SD)	0.03 (0.10)	0.02 (0.11)
Median	0.01	0.01
25th, 75th Percentile	-0.04, 0.09	-0.05, 0.09
Min, Max	-0.2, 0.3	-0.2, 0.3
LS mean change from baseline (95% CI)	0.02 (-0.02, 0.07)	0.02 (-0.02, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.05, 0.04)
P-value ^b		0.9133

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.013.001.000_mod_sub_eth_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.13.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.02 (-0.46, 0.41)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.013.001.000_mod_sub_eth_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.13.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	14
Mean (SD)	1.04 (0.06)	1.11 (0.11)
Median	1.05	1.09
25th, 75th Percentile	0.98, 1.09	1.05, 1.21
Min, Max	0.9, 1.1	0.9, 1.3
Week 52		
n	20	14
Mean (SD)	1.10 (0.09)	1.08 (0.10)
Median	1.09	1.06
25th, 75th Percentile	1.03, 1.18	1.02, 1.10
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.013.001.000_mod_sub_eth_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.13.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	14
Mean (SD)	0.06 (0.11)	-0.03 (0.07)
Median	0.03	-0.02
25th, 75th Percentile	-0.01, 0.11	-0.10, 0.01
Min, Max	-0.1, 0.3	-0.1, 0.1
LS mean change from baseline (95% CI)	0.07 (0.01, 0.12)	-0.03 (-0.09, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.10 (-0.17, -0.03)
P-value ^b		0.0060

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.13.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Ethnicity for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-1.10 (-1.87, -0.31)
P-value for interaction term, treatment * [Ethnicity]		0.0261

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.013.001.000_mod_sub_eth_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
North America		
Baseline		
n	26	27
Mean (SD)	1.08 (0.07)	1.10 (0.09)
Median	1.08	1.07
25th, 75th Percentile	1.03, 1.13	1.03, 1.15
Min, Max	1.0, 1.2	0.9, 1.3
Week 52		
n	26	27
Mean (SD)	1.09 (0.08)	1.07 (0.10)
Median	1.08	1.05
25th, 75th Percentile	1.03, 1.16	0.99, 1.12
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	26	27
Mean (SD)	0.01 (0.07)	-0.03 (0.07)
Median	0.01	-0.03
25th, 75th Percentile	-0.05, 0.06	-0.10, 0.02
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.05, 0.04)	-0.03 (-0.07, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.07, 0.01)
P-value ^b		0.1724

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.41 (-1.00, 0.18)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Europe		
Baseline		
n	18	17
Mean (SD)	1.02 (0.10)	1.05 (0.21)
Median	0.98	1.01
25th, 75th Percentile	0.96, 1.10	0.95, 1.09
Min, Max	0.9, 1.3	0.8, 1.6
Week 52		
n	18	17
Mean (SD)	1.05 (0.10)	1.09 (0.17)
Median	1.02	1.03
25th, 75th Percentile	0.99, 1.14	0.99, 1.09
Min, Max	0.9, 1.2	0.9, 1.5

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	17
Mean (SD)	0.04 (0.12)	0.03 (0.11)
Median	0.02	0.06
25th, 75th Percentile	-0.03, 0.09	-0.05, 0.10
Min, Max	-0.2, 0.3	-0.1, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.04, 0.11)	0.04 (-0.04, 0.12)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.08, 0.09)
P-value ^b		0.8956

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.05 (-0.63, 0.72)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	2
Mean (SD)	1.07 (0.08)	1.24 (0.01)
Median	1.11	1.24
25th, 75th Percentile	1.02, 1.13	1.24, 1.25
Min, Max	0.9, 1.1	1.2, 1.3
Week 52		
n	4	2
Mean (SD)	1.17 (0.10)	1.24 (0.02)
Median	1.19	1.24
25th, 75th Percentile	1.11, 1.23	1.23, 1.25
Min, Max	1.0, 1.3	1.2, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	4	2
Mean (SD)	0.10 (0.15)	-0.01 (0.03)
Median	0.12	-0.01
25th, 75th Percentile	-0.02, 0.22	-0.02, 0.01
Min, Max	-0.1, 0.2	0.0, 0.0
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
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NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Rest of World		
Baseline		
n	13	12
Mean (SD)	1.04 (0.05)	1.06 (0.06)
Median	1.05	1.05
25th, 75th Percentile	1.02, 1.09	1.01, 1.10
Min, Max	1.0, 1.1	0.9, 1.2
Week 52		
n	13	12
Mean (SD)	1.11 (0.07)	1.14 (0.12)
Median	1.11	1.09
25th, 75th Percentile	1.08, 1.14	1.04, 1.26
Min, Max	1.0, 1.2	1.0, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.4.14.1
Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	0.07 (0.10)	0.08 (0.12)
Median	0.08	0.03
25th, 75th Percentile	-0.01, 0.12	-0.02, 0.16
Min, Max	-0.1, 0.3	-0.1, 0.3
LS mean change from baseline (95% CI)	0.05 (-0.01, 0.11)	0.07 (0.01, 0.13)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.06, 0.10)
P-value ^b		0.6161

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.014.001.000_mod_sub_reg_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.4.14.1
 Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Arm Length to Lower Arm (Forearm) Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.22 (-0.64, 1.08)
P-value for interaction term, treatment * [Region]		0.4558

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper arm length to lower arm length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper arm length to lower arm length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.04.014.001.000_mod_sub_reg_armrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	30
Mean (SD)	0.65 (0.05)	0.64 (0.07)
Median	0.65	0.65
25th, 75th Percentile	0.61, 0.69	0.60, 0.70
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	33	30
Mean (SD)	0.66 (0.05)	0.65 (0.06)
Median	0.66	0.64
25th, 75th Percentile	0.63, 0.70	0.60, 0.69
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.007.001.000_mod_sub_sex_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	33	30
Mean (SD)	0.01 (0.06)	0.00 (0.06)
Median	0.01	0.02
25th, 75th Percentile	-0.03, 0.05	-0.04, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.02 (-0.01, 0.05)	0.01 (-0.02, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.04, 0.02)
P-value ^b		0.3975

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.007.001.000_mod_sub_sex_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.22 (-0.72, 0.29)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.007.001.000_mod_sub_sex_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	28
Mean (SD)	0.67 (0.05)	0.66 (0.06)
Median	0.67	0.65
25th, 75th Percentile	0.63, 0.70	0.62, 0.69
Min, Max	0.6, 0.8	0.5, 0.8
Week 52		
n	28	28
Mean (SD)	0.68 (0.05)	0.68 (0.07)
Median	0.68	0.67
25th, 75th Percentile	0.64, 0.71	0.64, 0.74
Min, Max	0.6, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.007.001.000_mod_sub_sex_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	28
Mean (SD)	0.01 (0.05)	0.02 (0.07)
Median	0.01	0.03
25th, 75th Percentile	-0.02, 0.03	-0.02, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.01, 0.04)	0.03 (0.00, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.02, 0.04)
P-value ^b		0.5129

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.007.001.000_mod_sub_sex_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.18 (-0.36, 0.73)
P-value for interaction term,treatment * [Sex]		0.3075

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.007.001.000_mod_sub_sex_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
≥ 5 to < 8		
Baseline		
n	24	31
Mean (SD)	0.65 (0.05)	0.63 (0.06)
Median	0.65	0.63
25th, 75th Percentile	0.61, 0.68	0.58, 0.66
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	24	31
Mean (SD)	0.66 (0.06)	0.65 (0.06)
Median	0.66	0.65
25th, 75th Percentile	0.63, 0.70	0.62, 0.68
Min, Max	0.5, 0.7	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	31
Mean (SD)	0.01 (0.06)	0.02 (0.07)
Median	0.02	0.04
25th, 75th Percentile	-0.03, 0.06	-0.03, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.01, 0.04)	0.02 (-0.01, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.03, 0.04)
P-value ^b		0.8447

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.05 (-0.49, 0.60)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	16
Mean (SD)	0.68 (0.05)	0.66 (0.06)
Median	0.68	0.65
25th, 75th Percentile	0.64, 0.71	0.61, 0.70
Min, Max	0.6, 0.8	0.6, 0.8
Week 52		
n	24	16
Mean (SD)	0.69 (0.04)	0.66 (0.07)
Median	0.69	0.64
25th, 75th Percentile	0.65, 0.72	0.58, 0.72
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	16
Mean (SD)	0.01 (0.04)	0.00 (0.06)
Median	0.01	0.00
25th, 75th Percentile	-0.02, 0.03	-0.04, 0.02
Min, Max	-0.1, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.01, 0.06)	0.00 (-0.03, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.2871

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.38 (-1.08, 0.32)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	11
Mean (SD)	0.65 (0.05)	0.70 (0.07)
Median	0.64	0.70
25th, 75th Percentile	0.61, 0.68	0.65, 0.74
Min, Max	0.6, 0.8	0.6, 0.8
Week 52		
n	13	11
Mean (SD)	0.67 (0.06)	0.71 (0.07)
Median	0.66	0.72
25th, 75th Percentile	0.62, 0.68	0.64, 0.76
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.02 (0.06)	0.01 (0.05)
Median	0.02	0.02
25th, 75th Percentile	-0.02, 0.05	-0.03, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	0.02 (-0.02, 0.06)	0.01 (-0.04, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.08, 0.06)
P-value ^b		0.7692

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.16 (-1.21, 0.89)
P-value for interaction term, treatment * [Age at Baseline]		0.7255

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.008.001.000_mod_sub_age_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	47
Mean (SD)	0.65 (0.05)	0.64 (0.06)
Median	0.66	0.65
25th, 75th Percentile	0.61, 0.69	0.60, 0.69
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	48	47
Mean (SD)	0.66 (0.05)	0.65 (0.06)
Median	0.67	0.65
25th, 75th Percentile	0.63, 0.70	0.61, 0.70
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.009.001.000_mod_sub_tan_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	48	47
Mean (SD)	0.01 (0.05)	0.01 (0.07)
Median	0.01	0.02
25th, 75th Percentile	-0.02, 0.05	-0.04, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	0.01 (0.00, 0.03)	0.01 (-0.01, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.03, 0.02)
P-value ^b		0.6065

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.009.001.000_mod_sub_tan_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.11 (-0.52, 0.30)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.009.001.000_mod_sub_tan_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	11
Mean (SD)	0.68 (0.05)	0.68 (0.07)
Median	0.68	0.66
25th, 75th Percentile	0.64, 0.72	0.62, 0.74
Min, Max	0.6, 0.8	0.6, 0.8
Week 52		
n	13	11
Mean (SD)	0.70 (0.06)	0.71 (0.08)
Median	0.69	0.73
25th, 75th Percentile	0.66, 0.72	0.61, 0.78
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.009.001.000_mod_sub_tan_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.01 (0.06)	0.03 (0.06)
Median	0.02	0.04
25th, 75th Percentile	-0.02, 0.03	-0.02, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.06)	0.02 (-0.03, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.07, 0.09)
P-value ^b		0.7703

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.16 (-0.92, 1.24)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5515

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.009.001.000_mod_sub_tan_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	27
Mean (SD)	0.65 (0.05)	0.65 (0.07)
Median	0.65	0.65
25th, 75th Percentile	0.60, 0.69	0.60, 0.70
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	28	27
Mean (SD)	0.66 (0.06)	0.65 (0.06)
Median	0.66	0.64
25th, 75th Percentile	0.63, 0.71	0.59, 0.69
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.010.001.000_mod_sub_strata_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	27
Mean (SD)	0.01 (0.06)	0.00 (0.06)
Median	0.01	0.02
25th, 75th Percentile	-0.02, 0.05	-0.04, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.01, 0.04)	0.00 (-0.03, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.05, 0.02)
P-value ^b		0.2875

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.010.001.000_mod_sub_strata_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.29 (-0.82, 0.24)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.010.001.000_mod_sub_strata_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	0.66 (0.05)	0.64 (0.05)
Median	0.66	0.64
25th, 75th Percentile	0.61, 0.69	0.61, 0.66
Min, Max	0.6, 0.8	0.5, 0.8
Week 52		
n	20	20
Mean (SD)	0.67 (0.04)	0.66 (0.06)
Median	0.68	0.65
25th, 75th Percentile	0.63, 0.70	0.63, 0.71
Min, Max	0.6, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	20
Mean (SD)	0.01 (0.04)	0.02 (0.07)
Median	0.01	0.03
25th, 75th Percentile	-0.02, 0.03	-0.02, 0.06
Min, Max	0.0, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.02, 0.04)	0.02 (0.00, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.02, 0.06)
P-value ^b		0.4317

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.010.001.000_mod_sub_strata_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.27 (-0.40, 0.94)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.010.001.000_mod_sub_strata_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	0.67 (0.06)	0.61 (0.04)
Median	0.67	0.60
25th, 75th Percentile	0.62, 0.68	0.59, 0.66
Min, Max	0.6, 0.8	0.6, 0.7
Week 52		
n	5	3
Mean (SD)	0.67 (0.06)	0.65 (0.06)
Median	0.67	0.61
25th, 75th Percentile	0.65, 0.69	0.61, 0.72
Min, Max	0.6, 0.7	0.6, 0.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	5	3
Mean (SD)	0.00 (0.06)	0.03 (0.03)
Median	0.00	0.02
25th, 75th Percentile	-0.03, 0.05	0.01, 0.06
Min, Max	-0.1, 0.1	0.0, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.17, 0.16)	0.04 (-0.21, 0.30)
Difference in LS mean change from baseline (95% CI) ^a		0.05 (-0.34, 0.43)
P-value ^b		0.7194

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.65 (-2.64, 3.84)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	8
Mean (SD)	0.69 (0.04)	0.70 (0.07)
Median	0.69	0.70
25th, 75th Percentile	0.65, 0.73	0.64, 0.74
Min, Max	0.6, 0.8	0.6, 0.8
Week 52		
n	8	8
Mean (SD)	0.71 (0.07)	0.73 (0.07)
Median	0.71	0.75
25th, 75th Percentile	0.67, 0.74	0.68, 0.79
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	8
Mean (SD)	0.02 (0.06)	0.03 (0.07)
Median	0.02	0.04
25th, 75th Percentile	-0.02, 0.03	-0.02, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.04, 0.08)	0.03 (-0.04, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.09, 0.10)
P-value ^b		0.9304

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.05 (-1.11, 1.21)
P-value for interaction term, treatment * [Strata]		0.6307

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	13
Mean (SD)	0.64 (0.05)	0.65 (0.05)
Median	0.64	0.64
25th, 75th Percentile	0.60, 0.69	0.62, 0.65
Min, Max	0.6, 0.7	0.6, 0.8
Week 52		
n	10	13
Mean (SD)	0.66 (0.05)	0.66 (0.06)
Median	0.67	0.65
25th, 75th Percentile	0.62, 0.70	0.64, 0.67
Min, Max	0.6, 0.7	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	10	13
Mean (SD)	0.02 (0.05)	0.01 (0.04)
Median	0.02	0.02
25th, 75th Percentile	-0.02, 0.03	-0.02, 0.05
Min, Max	0.0, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	0.00 (-0.04, 0.04)	0.01 (-0.02, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.04, 0.05)
P-value ^b		0.7549

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.15 (-0.78, 1.07)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	0.67 (0.05)	0.64 (0.08)
Median	0.67	0.64
25th, 75th Percentile	0.63, 0.69	0.58, 0.70
Min, Max	0.6, 0.8	0.5, 0.8
Week 52		
n	24	18
Mean (SD)	0.67 (0.05)	0.66 (0.08)
Median	0.67	0.66
25th, 75th Percentile	0.64, 0.71	0.60, 0.72
Min, Max	0.5, 0.7	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	18
Mean (SD)	0.00 (0.06)	0.02 (0.09)
Median	0.00	0.02
25th, 75th Percentile	-0.04, 0.04	-0.03, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.03, 0.04)	0.02 (-0.02, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		0.02 (-0.03, 0.07)
P-value ^b		0.4635

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.25 (-0.41, 0.90)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	0.66 (0.06)	0.65 (0.05)
Median	0.66	0.65
25th, 75th Percentile	0.63, 0.69	0.60, 0.70
Min, Max	0.5, 0.8	0.6, 0.7
Week 52		
n	19	22
Mean (SD)	0.68 (0.07)	0.66 (0.07)
Median	0.68	0.66
25th, 75th Percentile	0.66, 0.72	0.61, 0.71
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	22
Mean (SD)	0.02 (0.05)	0.01 (0.06)
Median	0.02	0.04
25th, 75th Percentile	-0.02, 0.05	-0.04, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	0.04 (0.00, 0.08)	0.02 (-0.01, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.3406

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.011.001.000_mod_sub_bhgt_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.33 (-1.00, 0.35)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	0.65 (0.06)	0.70 (0.09)
Median	0.64	0.68
25th, 75th Percentile	0.60, 0.70	0.64, 0.74
Min, Max	0.6, 0.7	0.6, 0.8
Week 52		
n	8	5
Mean (SD)	0.67 (0.06)	0.70 (0.05)
Median	0.67	0.70
25th, 75th Percentile	0.62, 0.72	0.69, 0.73
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	5
Mean (SD)	0.02 (0.05)	0.00 (0.05)
Median	0.01	0.01
25th, 75th Percentile	-0.02, 0.08	-0.04, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.0
LS mean change from baseline (95% CI)	0.02 (-0.08, 0.12)	0.01 (-0.06, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.14, 0.13)
P-value ^b		0.8732

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.18 (-2.23, 1.89)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.7456

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	18
Mean (SD)	0.67 (0.04)	0.66 (0.08)
Median	0.67	0.68
25th, 75th Percentile	0.63, 0.69	0.59, 0.72
Min, Max	0.6, 0.8	0.5, 0.8
Week 52		
n	19	18
Mean (SD)	0.68 (0.05)	0.65 (0.06)
Median	0.68	0.65
25th, 75th Percentile	0.64, 0.71	0.61, 0.71
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.012.001.000_mod_sub_bagv_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	18
Mean (SD)	0.01 (0.05)	-0.01 (0.07)
Median	0.00	0.00
25th, 75th Percentile	-0.02, 0.03	-0.05, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.05)	0.00 (-0.05, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.07, 0.03)
P-value ^b		0.5173

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.24 (-0.97, 0.49)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	0.66 (0.05)	0.65 (0.04)
Median	0.68	0.65
25th, 75th Percentile	0.61, 0.69	0.63, 0.68
Min, Max	0.6, 0.8	0.6, 0.7
Week 52		
n	18	14
Mean (SD)	0.68 (0.06)	0.67 (0.06)
Median	0.67	0.68
25th, 75th Percentile	0.63, 0.72	0.64, 0.72
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	14
Mean (SD)	0.02 (0.05)	0.02 (0.05)
Median	0.02	0.03
25th, 75th Percentile	-0.03, 0.05	-0.01, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	0.02 (-0.01, 0.05)	0.03 (-0.01, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.03, 0.05)
P-value ^b		0.7486

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.13 (-0.65, 0.90)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	26
Mean (SD)	0.65 (0.06)	0.64 (0.07)
Median	0.65	0.63
25th, 75th Percentile	0.60, 0.69	0.59, 0.66
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	24	26
Mean (SD)	0.66 (0.05)	0.66 (0.07)
Median	0.67	0.65
25th, 75th Percentile	0.63, 0.70	0.61, 0.72
Min, Max	0.5, 0.7	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	26
Mean (SD)	0.01 (0.06)	0.02 (0.07)
Median	0.01	0.02
25th, 75th Percentile	-0.03, 0.04	-0.02, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.03, 0.06)	0.03 (-0.01, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (-0.04, 0.05)
P-value ^b		0.7397

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.12.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.11 (-0.54, 0.76)
P-value for interaction term, treatment * [Baseline AGV]		0.6958

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	44
Mean (SD)	0.66 (0.05)	0.65 (0.07)
Median	0.66	0.64
25th, 75th Percentile	0.62, 0.69	0.60, 0.70
Min, Max	0.6, 0.8	0.5, 0.8
Week 52		
n	41	44
Mean (SD)	0.67 (0.04)	0.66 (0.07)
Median	0.67	0.65
25th, 75th Percentile	0.64, 0.69	0.62, 0.72
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.013.001.000_mod_sub_eth_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	41	44
Mean (SD)	0.01 (0.05)	0.01 (0.06)
Median	0.00	0.02
25th, 75th Percentile	-0.02, 0.05	-0.03, 0.05
Min, Max	-0.1, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.01, 0.04)	0.02 (0.00, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.03, 0.02)
P-value ^b		0.9654

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.013.001.000_mod_sub_eth_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.01 (-0.45, 0.43)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.013.001.000_mod_sub_eth_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	14
Mean (SD)	0.66 (0.05)	0.65 (0.06)
Median	0.67	0.65
25th, 75th Percentile	0.60, 0.69	0.62, 0.66
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	20	14
Mean (SD)	0.68 (0.07)	0.67 (0.06)
Median	0.68	0.68
25th, 75th Percentile	0.64, 0.73	0.60, 0.72
Min, Max	0.5, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.013.001.000_mod_sub_eth_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	14
Mean (SD)	0.02 (0.06)	0.02 (0.09)
Median	0.02	0.03
25th, 75th Percentile	-0.02, 0.05	-0.04, 0.06
Min, Max	-0.1, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.04, 0.05)	0.00 (-0.04, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.05, 0.06)
P-value ^b		0.9365

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.013.001.000_mod_sub_eth_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.03 (-0.69, 0.75)
P-value for interaction term, treatment * [Ethnicity]		0.7392

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.013.001.000_mod_sub_eth_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
North America		
Baseline		
n	26	27
Mean (SD)	0.66 (0.06)	0.65 (0.08)
Median	0.67	0.65
25th, 75th Percentile	0.61, 0.69	0.59, 0.70
Min, Max	0.5, 0.8	0.5, 0.8
Week 52		
n	26	27
Mean (SD)	0.67 (0.07)	0.65 (0.07)
Median	0.67	0.64
25th, 75th Percentile	0.63, 0.72	0.60, 0.72
Min, Max	0.5, 0.8	0.5, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.014.001.000_mod_sub_reg_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	26	27
Mean (SD)	0.01 (0.06)	0.00 (0.07)
Median	0.01	-0.01
25th, 75th Percentile	-0.04, 0.05	-0.07, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.05)	0.00 (-0.03, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.05, 0.03)
P-value ^b		0.5784

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.014.001.000_mod_sub_reg_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.17 (-0.75, 0.42)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Europe		
Baseline		
n	18	17
Mean (SD)	0.65 (0.04)	0.64 (0.05)
Median	0.65	0.64
25th, 75th Percentile	0.62, 0.68	0.60, 0.69
Min, Max	0.6, 0.7	0.5, 0.7
Week 52		
n	18	17
Mean (SD)	0.67 (0.03)	0.67 (0.06)
Median	0.68	0.68
25th, 75th Percentile	0.66, 0.70	0.64, 0.71
Min, Max	0.6, 0.7	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	17
Mean (SD)	0.03 (0.04)	0.03 (0.07)
Median	0.02	0.02
25th, 75th Percentile	0.00, 0.05	-0.03, 0.05
Min, Max	0.0, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.01, 0.07)	0.04 (-0.01, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.04, 0.05)
P-value ^b		0.8632

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.06 (-0.61, 0.73)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	2
Mean (SD)	0.65 (0.04)	0.67 (0.12)
Median	0.65	0.67
25th, 75th Percentile	0.62, 0.69	0.58, 0.75
Min, Max	0.6, 0.7	0.6, 0.8
Week 52		
n	4	2
Mean (SD)	0.65 (0.05)	0.69 (0.07)
Median	0.64	0.69
25th, 75th Percentile	0.61, 0.69	0.64, 0.74
Min, Max	0.6, 0.7	0.6, 0.7

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.014.001.000_mod_sub_reg_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	4	2
Mean (SD)	0.00 (0.03)	0.03 (0.05)
Median	0.01	0.03
25th, 75th Percentile	-0.02, 0.02	-0.01, 0.07
Min, Max	0.0, 0.0	0.0, 0.1
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

<u>Upper Leg Length (Thigh) to Knee to Heel Length Ratio</u>	<u>Placebo (N=61)</u>	<u>15 ug/kg BMN 111 (N=60)</u>
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NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Rest of World		
Baseline		
n	13	12
Mean (SD)	0.67 (0.06)	0.65 (0.05)
Median	0.68	0.64
25th, 75th Percentile	0.64, 0.71	0.63, 0.69
Min, Max	0.6, 0.8	0.6, 0.7
Week 52		
n	13	12
Mean (SD)	0.68 (0.06)	0.68 (0.07)
Median	0.68	0.67
25th, 75th Percentile	0.65, 0.72	0.63, 0.72
Min, Max	0.6, 0.8	0.6, 0.8

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.014.001.000_mod_sub_reg_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	0.01 (0.05)	0.02 (0.03)
Median	0.00	0.03
25th, 75th Percentile	-0.03, 0.05	0.01, 0.05
Min, Max	-0.1, 0.1	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.03)	0.03 (0.00, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.03 (-0.01, 0.06)
P-value ^b		0.1198

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.014.001.000_mod_sub_reg_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.5.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Knee to Heel Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.72 (-0.18, 1.60)
P-value for interaction term,treatment * [Region]		0.7366

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to knee to heel length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to knee to heel length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.05.014.001.000_mod_sub_reg_legrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	30
Mean (SD)	1.07 (0.11)	1.07 (0.13)
Median	1.07	1.06
25th, 75th Percentile	1.02, 1.13	0.97, 1.15
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	33	30
Mean (SD)	1.08 (0.09)	1.05 (0.12)
Median	1.08	1.03
25th, 75th Percentile	1.01, 1.12	0.97, 1.13
Min, Max	0.9, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.007.001.000_mod_sub_sex_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	33	30
Mean (SD)	0.01 (0.08)	-0.01 (0.09)
Median	0.02	0.02
25th, 75th Percentile	-0.04, 0.07	-0.07, 0.06
Min, Max	-0.2, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.01, 0.07)	0.01 (-0.04, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.07, 0.03)
P-value ^b		0.4002

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.007.001.000_mod_sub_sex_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.7.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Sex for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.22 (-0.72, 0.29)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.007.001.000_mod_sub_sex_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	28
Mean (SD)	1.09 (0.12)	1.07 (0.13)
Median	1.10	1.04
25th, 75th Percentile	1.01, 1.17	0.97, 1.17
Min, Max	0.9, 1.4	0.9, 1.5
Week 52		
n	28	28
Mean (SD)	1.12 (0.13)	1.08 (0.14)
Median	1.11	1.03
25th, 75th Percentile	1.01, 1.19	0.99, 1.23
Min, Max	0.9, 1.4	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.007.001.000_mod_sub_sex_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	28
Mean (SD)	0.03 (0.08)	0.01 (0.11)
Median	0.02	0.02
25th, 75th Percentile	-0.01, 0.07	-0.06, 0.09
Min, Max	-0.1, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.01, 0.07)	0.02 (-0.02, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.06, 0.04)
P-value ^b		0.6564

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.007.001.000_mod_sub_sex_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.7.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.12 (-0.67, 0.42)
P-value for interaction term, treatment * [Sex]		0.9360

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.007.001.000_mod_sub_sex_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Baseline		
n	24	31
Mean (SD)	1.05 (0.10)	1.05 (0.14)
Median	1.05	1.01
25th, 75th Percentile	0.98, 1.12	0.96, 1.15
Min, Max	0.9, 1.3	0.8, 1.5
Week 52		
n	24	31
Mean (SD)	1.06 (0.08)	1.05 (0.12)
Median	1.04	1.03
25th, 75th Percentile	1.00, 1.14	0.97, 1.11
Min, Max	0.9, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	31
Mean (SD)	0.01 (0.09)	0.00 (0.10)
Median	0.01	0.02
25th, 75th Percentile	-0.03, 0.06	-0.05, 0.07
Min, Max	-0.2, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.05)	0.00 (-0.03, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.06, 0.05)
P-value ^b		0.8949

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.04 (-0.58, 0.51)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	16
Mean (SD)	1.11 (0.11)	1.07 (0.11)
Median	1.11	1.06
25th, 75th Percentile	1.04, 1.16	1.01, 1.13
Min, Max	0.9, 1.4	0.9, 1.3
Week 52		
n	24	16
Mean (SD)	1.12 (0.11)	1.05 (0.13)
Median	1.09	1.01
25th, 75th Percentile	1.05, 1.17	0.94, 1.16
Min, Max	0.9, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	16
Mean (SD)	0.01 (0.06)	-0.02 (0.12)
Median	0.01	-0.04
25th, 75th Percentile	-0.05, 0.05	-0.12, 0.04
Min, Max	-0.1, 0.1	-0.2, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.05, 0.08)	-0.01 (-0.07, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.10, 0.04)
P-value ^b		0.4203

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.29 (-0.99, 0.41)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	11
Mean (SD)	1.07 (0.12)	1.11 (0.13)
Median	1.06	1.10
25th, 75th Percentile	1.02, 1.12	1.06, 1.19
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	13	11
Mean (SD)	1.13 (0.16)	1.14 (0.15)
Median	1.08	1.18
25th, 75th Percentile	1.02, 1.20	1.02, 1.27
Min, Max	1.0, 1.4	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.06 (0.09)	0.03 (0.06)
Median	0.06	0.05
25th, 75th Percentile	0.01, 0.09	-0.03, 0.08
Min, Max	-0.1, 0.2	-0.1, 0.1
LS mean change from baseline (95% CI)	0.06 (0.01, 0.11)	0.04 (-0.02, 0.11)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.10, 0.07)
P-value ^b		0.7212

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.8.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.19 (-1.24, 0.86)
P-value for interaction term, treatment * [Age at Baseline]		0.7615

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.008.001.000_mod_sub_age_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	47
Mean (SD)	1.06 (0.10)	1.06 (0.13)
Median	1.07	1.05
25th, 75th Percentile	0.99, 1.13	0.97, 1.15
Min, Max	0.9, 1.3	0.8, 1.5
Week 52		
n	48	47
Mean (SD)	1.08 (0.09)	1.05 (0.11)
Median	1.07	1.03
25th, 75th Percentile	1.01, 1.13	0.98, 1.11
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.009.001.000_mod_sub_tan_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	48	47
Mean (SD)	0.02 (0.08)	-0.01 (0.10)
Median	0.01	-0.02
25th, 75th Percentile	-0.03, 0.06	-0.07, 0.06
Min, Max	-0.2, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.01, 0.05)	-0.01 (-0.04, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.07, 0.00)
P-value ^b		0.0728

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.009.001.000_mod_sub_tan_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.9.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.38 (-0.79, 0.03)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.009.001.000_mod_sub_tan_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	11
Mean (SD)	1.14 (0.15)	1.08 (0.14)
Median	1.13	1.09
25th, 75th Percentile	1.06, 1.22	0.96, 1.19
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	13	11
Mean (SD)	1.17 (0.15)	1.15 (0.18)
Median	1.15	1.21
25th, 75th Percentile	1.08, 1.24	0.93, 1.29
Min, Max	1.0, 1.4	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.009.001.000_mod_sub_tan_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.03 (0.08)	0.07 (0.09)
Median	0.03	0.06
25th, 75th Percentile	-0.04, 0.07	0.02, 0.09
Min, Max	-0.1, 0.2	-0.1, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.04, 0.08)	0.06 (-0.01, 0.13)
Difference in LS mean change from baseline (95% CI) ^a		0.04 (-0.06, 0.14)
P-value ^b		0.4647

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.009.001.000_mod_sub_tan_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.9.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.41 (-0.68, 1.49)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.0719

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.009.001.000_mod_sub_tan_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	27
Mean (SD)	1.06 (0.09)	1.08 (0.12)
Median	1.07	1.06
25th, 75th Percentile	1.00, 1.13	0.98, 1.18
Min, Max	0.9, 1.2	0.8, 1.3
Week 52		
n	28	27
Mean (SD)	1.06 (0.07)	1.06 (0.11)
Median	1.07	1.03
25th, 75th Percentile	1.01, 1.12	0.98, 1.13
Min, Max	0.9, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.010.001.000_mod_sub_strata_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	27
Mean (SD)	0.01 (0.09)	-0.02 (0.10)
Median	0.02	-0.02
25th, 75th Percentile	-0.06, 0.06	-0.07, 0.06
Min, Max	-0.2, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.01 (-0.03, 0.04)	-0.02 (-0.05, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.08, 0.03)
P-value ^b		0.3239

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.010.001.000_mod_sub_strata_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.27 (-0.80, 0.26)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.010.001.000_mod_sub_strata_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	1.06 (0.11)	1.05 (0.14)
Median	1.08	1.00
25th, 75th Percentile	0.99, 1.13	0.96, 1.09
Min, Max	0.9, 1.3	0.9, 1.5
Week 52		
n	20	20
Mean (SD)	1.10 (0.12)	1.03 (0.11)
Median	1.09	1.01
25th, 75th Percentile	1.00, 1.16	0.97, 1.04
Min, Max	0.9, 1.3	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.010.001.000_mod_sub_strata_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	20
Mean (SD)	0.03 (0.07)	-0.01 (0.10)
Median	0.01	-0.02
25th, 75th Percentile	0.00, 0.07	-0.08, 0.07
Min, Max	-0.1, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.01, 0.08)	-0.01 (-0.05, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.04 (-0.11, 0.02)
P-value ^b		0.1553

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.010.001.000_mod_sub_strata_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.49 (-1.17, 0.19)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.010.001.000_mod_sub_strata_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	1.11 (0.17)	0.95 (0.13)
Median	1.12	0.90
25th, 75th Percentile	1.06, 1.13	0.84, 1.09
Min, Max	0.9, 1.4	0.8, 1.1
Week 52		
n	5	3
Mean (SD)	1.15 (0.16)	0.99 (0.17)
Median	1.11	0.92
25th, 75th Percentile	1.08, 1.20	0.87, 1.18
Min, Max	1.0, 1.4	0.9, 1.2

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	5	3
Mean (SD)	0.04 (0.05)	0.04 (0.04)
Median	0.06	0.02
25th, 75th Percentile	0.02, 0.07	0.01, 0.09
Min, Max	0.0, 0.1	0.0, 0.1
LS mean change from baseline (95% CI)	0.04 (-0.07, 0.15)	0.04 (-0.13, 0.20)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.26, 0.25)
P-value ^b		0.9713

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.06 (-3.26, 3.14)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	8
Mean (SD)	1.17 (0.14)	1.13 (0.12)
Median	1.17	1.13
25th, 75th Percentile	1.04, 1.26	1.05, 1.21
Min, Max	1.0, 1.4	1.0, 1.3
Week 52		
n	8	8
Mean (SD)	1.19 (0.16)	1.20 (0.15)
Median	1.16	1.26
25th, 75th Percentile	1.09, 1.31	1.11, 1.29
Min, Max	1.0, 1.4	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	8
Mean (SD)	0.02 (0.10)	0.07 (0.11)
Median	0.02	0.07
25th, 75th Percentile	-0.06, 0.06	0.03, 0.13
Min, Max	-0.1, 0.2	-0.1, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.06, 0.12)	0.07 (-0.02, 0.16)
Difference in LS mean change from baseline (95% CI) ^a		0.04 (-0.10, 0.18)
P-value ^b		0.5461

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.10.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.37 (-0.81, 1.53)
P-value for interaction term, treatment * [Strata]		0.2941

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	13
Mean (SD)	1.03 (0.09)	1.08 (0.17)
Median	1.06	1.06
25th, 75th Percentile	0.92, 1.12	0.97, 1.09
Min, Max	0.9, 1.1	0.9, 1.5
Week 52		
n	10	13
Mean (SD)	1.06 (0.09)	1.04 (0.14)
Median	1.05	1.01
25th, 75th Percentile	1.00, 1.12	0.94, 1.05
Min, Max	0.9, 1.2	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	10	13
Mean (SD)	0.03 (0.05)	-0.03 (0.10)
Median	0.02	-0.03
25th, 75th Percentile	0.00, 0.08	-0.12, 0.05
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	0.01 (-0.07, 0.09)	-0.04 (-0.10, 0.03)
Difference in LS mean change from baseline (95% CI) ^a		-0.04 (-0.13, 0.05)
P-value ^b		0.3324

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.47 (-1.40, 0.47)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	1.08 (0.11)	1.06 (0.12)
Median	1.08	1.06
25th, 75th Percentile	1.01, 1.13	1.00, 1.15
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	24	18
Mean (SD)	1.10 (0.10)	1.07 (0.12)
Median	1.08	1.03
25th, 75th Percentile	1.03, 1.16	0.99, 1.13
Min, Max	1.0, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	18
Mean (SD)	0.02 (0.09)	0.00 (0.13)
Median	0.02	-0.01
25th, 75th Percentile	-0.05, 0.07	-0.09, 0.07
Min, Max	-0.1, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.03, 0.08)	0.02 (-0.05, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.08, 0.07)
P-value ^b		0.8711

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

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Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.05 (-0.71, 0.60)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	1.10 (0.13)	1.07 (0.12)
Median	1.09	1.05
25th, 75th Percentile	1.02, 1.19	0.97, 1.18
Min, Max	0.9, 1.4	0.9, 1.3
Week 52		
n	19	22
Mean (SD)	1.15 (0.14)	1.08 (0.13)
Median	1.13	1.03
25th, 75th Percentile	1.05, 1.21	0.98, 1.18
Min, Max	0.9, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	22
Mean (SD)	0.04 (0.08)	0.01 (0.07)
Median	0.02	0.03
25th, 75th Percentile	0.00, 0.13	-0.03, 0.07
Min, Max	-0.1, 0.2	-0.2, 0.1
LS mean change from baseline (95% CI)	0.04 (-0.01, 0.10)	0.02 (-0.03, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.03 (-0.08, 0.03)
P-value ^b		0.2850

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.011.001.000_mod_sub_bhgt_legtrt_301_fas.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.37 (-1.04, 0.31)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.011.001.000_mod_sub_bhgt_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	1.07 (0.11)	1.04 (0.13)
Median	1.11	1.10
25th, 75th Percentile	1.01, 1.15	0.98, 1.10
Min, Max	0.9, 1.2	0.8, 1.2
Week 52		
n	8	5
Mean (SD)	1.04 (0.08)	1.07 (0.16)
Median	1.03	1.03
25th, 75th Percentile	0.98, 1.10	1.00, 1.21
Min, Max	1.0, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.011.001.000_mod_sub_bhgt_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	5
Mean (SD)	-0.03 (0.09)	0.03 (0.08)
Median	-0.02	0.02
25th, 75th Percentile	-0.08, 0.04	0.02, 0.02
Min, Max	-0.2, 0.1	-0.1, 0.2
LS mean change from baseline (95% CI)	-0.02 (-0.12, 0.08)	0.07 (0.00, 0.14)
Difference in LS mean change from baseline (95% CI) ^a		0.09 (-0.04, 0.23)
P-value ^b		0.1380

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.011.001.000_mod_sub_bhgt_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.11.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.85 (-0.56, 4.12)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.3131

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.011.001.000_mod_sub_bhgt_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	18
Mean (SD)	1.11 (0.11)	1.09 (0.14)
Median	1.12	1.08
25th, 75th Percentile	1.03, 1.15	1.00, 1.18
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	19	18
Mean (SD)	1.13 (0.12)	1.06 (0.11)
Median	1.12	1.03
25th, 75th Percentile	1.08, 1.16	0.99, 1.10
Min, Max	1.0, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	18
Mean (SD)	0.02 (0.07)	-0.03 (0.12)
Median	0.03	-0.05
25th, 75th Percentile	-0.04, 0.07	-0.10, 0.07
Min, Max	-0.1, 0.1	-0.3, 0.2
LS mean change from baseline (95% CI)	0.02 (-0.05, 0.08)	-0.03 (-0.11, 0.04)
Difference in LS mean change from baseline (95% CI) ^a		-0.05 (-0.13, 0.03)
P-value ^b		0.2055

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.48 (-1.22, 0.26)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	1.09 (0.13)	1.06 (0.10)
Median	1.10	1.07
25th, 75th Percentile	1.02, 1.15	0.97, 1.10
Min, Max	0.9, 1.4	0.9, 1.2
Week 52		
n	18	14
Mean (SD)	1.12 (0.14)	1.08 (0.13)
Median	1.09	1.04
25th, 75th Percentile	1.02, 1.17	1.00, 1.13
Min, Max	0.9, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	14
Mean (SD)	0.03 (0.09)	0.01 (0.10)
Median	0.02	0.02
25th, 75th Percentile	-0.04, 0.06	-0.05, 0.08
Min, Max	-0.1, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.02, 0.09)	0.03 (-0.03, 0.09)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.08, 0.07)
P-value ^b		0.9316

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.03 (-0.80, 0.74)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	26
Mean (SD)	1.04 (0.10)	1.06 (0.14)
Median	1.04	1.04
25th, 75th Percentile	0.96, 1.12	0.96, 1.15
Min, Max	0.9, 1.2	0.8, 1.5
Week 52		
n	24	26
Mean (SD)	1.06 (0.09)	1.07 (0.15)
Median	1.05	1.02
25th, 75th Percentile	1.00, 1.11	0.94, 1.18
Min, Max	0.9, 1.2	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	26
Mean (SD)	0.02 (0.09)	0.01 (0.09)
Median	0.02	0.02
25th, 75th Percentile	-0.03, 0.09	-0.03, 0.06
Min, Max	-0.2, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.03 (-0.04, 0.09)	0.02 (-0.02, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.07, 0.06)
P-value ^b		0.8695

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.12.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.06 (-0.71, 0.60)
P-value for interaction term, treatment * [Baseline AGV]		0.7825

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.012.001.000_mod_sub_bagv_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	44
Mean (SD)	1.08 (0.12)	1.06 (0.12)
Median	1.07	1.05
25th, 75th Percentile	1.02, 1.14	0.96, 1.15
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	41	44
Mean (SD)	1.11 (0.12)	1.07 (0.13)
Median	1.09	1.03
25th, 75th Percentile	1.02, 1.16	0.99, 1.16
Min, Max	0.9, 1.4	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.013.001.000_mod_sub_eth_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	41	44
Mean (SD)	0.03 (0.08)	0.01 (0.09)
Median	0.03	0.02
25th, 75th Percentile	0.00, 0.07	-0.05, 0.06
Min, Max	-0.1, 0.2	-0.2, 0.2
LS mean change from baseline (95% CI)	0.04 (0.01, 0.07)	0.02 (-0.01, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.3133

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.013.001.000_mod_sub_eth_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.13.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Ethnicity for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.23 (-0.66, 0.21)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.013.001.000_mod_sub_eth_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	14
Mean (SD)	1.07 (0.10)	1.10 (0.15)
Median	1.10	1.08
25th, 75th Percentile	0.99, 1.15	0.98, 1.20
Min, Max	0.9, 1.3	0.9, 1.5
Week 52		
n	20	14
Mean (SD)	1.08 (0.10)	1.08 (0.13)
Median	1.07	1.02
25th, 75th Percentile	1.00, 1.13	0.97, 1.18
Min, Max	1.0, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.013.001.000_mod_sub_eth_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.13.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	14
Mean (SD)	0.01 (0.09)	-0.02 (0.13)
Median	0.00	0.01
25th, 75th Percentile	-0.04, 0.06	-0.14, 0.09
Min, Max	-0.2, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.00 (-0.07, 0.07)	-0.01 (-0.09, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.10, 0.08)
P-value ^b		0.8012

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.13.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Ethnicity for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.09 (-0.82, 0.63)
P-value for interaction term, treatment * [Ethnicity]		0.8246

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.013.001.000_mod_sub_eth_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
North America		
Baseline		
n	26	27
Mean (SD)	1.10 (0.10)	1.07 (0.12)
Median	1.10	1.06
25th, 75th Percentile	1.02, 1.15	1.00, 1.18
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	26	27
Mean (SD)	1.11 (0.10)	1.06 (0.12)
Median	1.10	1.02
25th, 75th Percentile	1.04, 1.16	0.97, 1.18
Min, Max	1.0, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	26	27
Mean (SD)	0.01 (0.08)	-0.01 (0.12)
Median	0.01	-0.03
25th, 75th Percentile	-0.06, 0.06	-0.09, 0.09
Min, Max	-0.1, 0.2	-0.3, 0.2
LS mean change from baseline (95% CI)	0.04 (-0.02, 0.10)	0.01 (-0.04, 0.06)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.09, 0.04)
P-value ^b		0.4711

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.22 (-0.80, 0.37)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.014.001.000_mod_sub_reg_legtrt_301_fas.pdf+rtf

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Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Europe		
Baseline		
n	18	17
Mean (SD)	1.07 (0.11)	1.06 (0.12)
Median	1.07	1.08
25th, 75th Percentile	0.99, 1.12	0.96, 1.15
Min, Max	0.9, 1.4	0.8, 1.3
Week 52		
n	18	17
Mean (SD)	1.09 (0.14)	1.06 (0.12)
Median	1.07	1.03
25th, 75th Percentile	0.99, 1.15	0.99, 1.12
Min, Max	0.9, 1.4	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	17
Mean (SD)	0.02 (0.09)	0.00 (0.07)
Median	0.03	0.02
25th, 75th Percentile	-0.02, 0.06	-0.05, 0.06
Min, Max	-0.2, 0.2	-0.1, 0.1
LS mean change from baseline (95% CI)	0.03 (-0.03, 0.09)	0.01 (-0.06, 0.07)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.09, 0.04)
P-value ^b		0.4451

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.27 (-0.94, 0.41)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	2
Mean (SD)	1.04 (0.17)	1.17 (0.42)
Median	1.01	1.17
25th, 75th Percentile	0.93, 1.15	0.88, 1.47
Min, Max	0.9, 1.3	0.9, 1.5
Week 52		
n	4	2
Mean (SD)	1.03 (0.09)	1.12 (0.26)
Median	1.00	1.12
25th, 75th Percentile	0.97, 1.09	0.94, 1.31
Min, Max	1.0, 1.2	0.9, 1.3

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	4	2
Mean (SD)	-0.01 (0.08)	-0.05 (0.16)
Median	-0.01	-0.05
25th, 75th Percentile	-0.07, 0.05	-0.16, 0.06
Min, Max	-0.1, 0.1	-0.2, 0.1
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
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NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Rest of World		
Baseline		
n	13	12
Mean (SD)	1.07 (0.13)	1.06 (0.13)
Median	1.09	1.00
25th, 75th Percentile	0.94, 1.17	0.96, 1.16
Min, Max	0.9, 1.2	0.9, 1.3
Week 52		
n	13	12
Mean (SD)	1.10 (0.11)	1.09 (0.16)
Median	1.08	1.04
25th, 75th Percentile	1.05, 1.18	0.97, 1.25
Min, Max	0.9, 1.3	0.9, 1.4

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.014.001.000_mod_sub_reg_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	0.04 (0.07)	0.03 (0.05)
Median	0.03	0.04
25th, 75th Percentile	0.01, 0.09	-0.02, 0.08
Min, Max	-0.1, 0.1	-0.1, 0.1
LS mean change from baseline (95% CI)	0.02 (-0.02, 0.07)	0.02 (-0.03, 0.08)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.06, 0.06)
P-value ^b		0.9588

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.014.001.000_mod_sub_reg_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.6.14.1
 Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Upper Leg Length (Thigh) to Tibial Leg Length Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.02 (-0.83, 0.88)
P-value for interaction term, treatment * [Region]		0.9665

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline upper leg length to tibial leg length ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline upper leg length to tibial leg length ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.06.014.001.000_mod_sub_reg_legtrt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.7.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Baseline		
n	33	30
Mean (SD)	0.90 (0.03)	0.90 (0.04)
Median	0.90	0.90
25th, 75th Percentile	0.89, 0.92	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	33	30
Mean (SD)	0.90 (0.04)	0.90 (0.04)
Median	0.90	0.90
25th, 75th Percentile	0.89, 0.93	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.007.001.000_mod_sub_sex_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.7.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	33	30
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.01)
P-value ^b		0.6349

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.007.001.000_mod_sub_sex_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.7.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.12 (-0.62, 0.38)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.007.001.000_mod_sub_sex_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.7.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Baseline		
n	28	28
Mean (SD)	0.89 (0.05)	0.90 (0.07)
Median	0.89	0.90
25th, 75th Percentile	0.85, 0.93	0.88, 0.92
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	28	28
Mean (SD)	0.90 (0.05)	0.89 (0.04)
Median	0.90	0.89
25th, 75th Percentile	0.85, 0.93	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.007.001.000_mod_sub_sex_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.7.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Sex for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	28
Mean (SD)	0.00 (0.02)	-0.01 (0.06)
Median	0.01	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.02)	-0.01 (-0.03, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.04, 0.01)
P-value ^b		0.2553

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.007.001.000_mod_sub_sex_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.7.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Sex for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.32 (-0.87, 0.23)
P-value for interaction term, treatment * [Sex]		0.2137

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and sex interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.007.001.000_mod_sub_sex_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Baseline		
n	24	31
Mean (SD)	0.90 (0.04)	0.91 (0.07)
Median	0.90	0.91
25th, 75th Percentile	0.87, 0.92	0.87, 0.93
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	24	31
Mean (SD)	0.90 (0.04)	0.90 (0.04)
Median	0.90	0.91
25th, 75th Percentile	0.87, 0.92	0.87, 0.93
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	31
Mean (SD)	0.00 (0.01)	-0.01 (0.06)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.02)	-0.01 (-0.02, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.03, 0.02)
P-value ^b		0.5179

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.18 (-0.73, 0.37)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Baseline		
n	24	16
Mean (SD)	0.90 (0.05)	0.89 (0.03)
Median	0.90	0.89
25th, 75th Percentile	0.88, 0.93	0.86, 0.90
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	24	16
Mean (SD)	0.90 (0.05)	0.89 (0.04)
Median	0.90	0.88
25th, 75th Percentile	0.87, 0.93	0.86, 0.90
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	16
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.01 (0.00, 0.02)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.02, 0.00)
P-value ^b		0.1168

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.57 (-1.28, 0.14)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Baseline		
n	13	11
Mean (SD)	0.90 (0.04)	0.89 (0.04)
Median	0.89	0.90
25th, 75th Percentile	0.87, 0.93	0.88, 0.92
Min, Max	0.8, 1.0	0.8, 0.9
Week 52		
n	13	11
Mean (SD)	0.90 (0.04)	0.89 (0.04)
Median	0.90	0.90
25th, 75th Percentile	0.87, 0.92	0.88, 0.92
Min, Max	0.8, 1.0	0.8, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.00 (0.02)	0.00 (0.01)
Median	0.01	-0.01
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.02, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.02, 0.01)
P-value ^b		0.4947

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.8.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Age at Baseline for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.37 (-1.42, 0.69)
P-value for interaction term, treatment * [Age at Baseline]		0.8808

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and age interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.008.001.000_mod_sub_age_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.9.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Baseline		
n	48	47
Mean (SD)	0.90 (0.04)	0.90 (0.06)
Median	0.89	0.90
25th, 75th Percentile	0.87, 0.92	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	48	47
Mean (SD)	0.90 (0.04)	0.89 (0.04)
Median	0.90	0.89
25th, 75th Percentile	0.87, 0.93	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.009.001.000_mod_sub_tan_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.9.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	48	47
Mean (SD)	0.00 (0.01)	-0.01 (0.05)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	-0.01 (-0.02, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		-0.01 (-0.02, 0.01)
P-value ^b		0.2246

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.009.001.000_mod_sub_tan_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.9.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.26 (-0.66, 0.16)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.009.001.000_mod_sub_tan_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.9.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Baseline		
n	13	11
Mean (SD)	0.91 (0.05)	0.90 (0.04)
Median	0.91	0.90
25th, 75th Percentile	0.87, 0.93	0.86, 0.93
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	13	11
Mean (SD)	0.91 (0.05)	0.90 (0.04)
Median	0.91	0.90
25th, 75th Percentile	0.88, 0.93	0.87, 0.93
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.009.001.000_mod_sub_tan_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.9.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	11
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.00)	0.00 (0.00, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (0.00, 0.02)
P-value ^b		0.2569

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.009.001.000_mod_sub_tan_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.9.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Tanner Stage for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.64 (-0.46, 1.74)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5821

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and tanner stage interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.009.001.000_mod_sub_tan_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage I		
Baseline		
n	28	27
Mean (SD)	0.90 (0.04)	0.90 (0.04)
Median	0.90	0.90
25th, 75th Percentile	0.89, 0.93	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	28	27
Mean (SD)	0.91 (0.04)	0.90 (0.04)
Median	0.90	0.89
25th, 75th Percentile	0.88, 0.93	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.010.001.000_mod_sub_strata_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	28	27
Mean (SD)	0.00 (0.01)	0.00 (0.02)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (0.00, 0.01)	0.00 (0.00, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.01)
P-value ^b		0.9020

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.010.001.000_mod_sub_strata_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.03 (-0.56, 0.50)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.010.001.000_mod_sub_strata_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage I		
Baseline		
n	20	20
Mean (SD)	0.88 (0.05)	0.91 (0.08)
Median	0.88	0.90
25th, 75th Percentile	0.85, 0.92	0.88, 0.92
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	20	20
Mean (SD)	0.89 (0.04)	0.89 (0.04)
Median	0.89	0.90
25th, 75th Percentile	0.85, 0.92	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.010.001.000_mod_sub_strata_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	20
Mean (SD)	0.00 (0.02)	-0.02 (0.07)
Median	0.01	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.02, 0.03)	-0.02 (-0.04, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.2614

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.39 (-1.06, 0.29)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.010.001.000_mod_sub_strata_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male Tanner Stage > I		
Baseline		
n	5	3
Mean (SD)	0.89 (0.03)	0.91 (0.05)
Median	0.89	0.93
25th, 75th Percentile	0.87, 0.91	0.86, 0.95
Min, Max	0.9, 0.9	0.9, 0.9
Week 52		
n	5	3
Mean (SD)	0.90 (0.03)	0.91 (0.03)
Median	0.90	0.92
25th, 75th Percentile	0.89, 0.92	0.87, 0.94
Min, Max	0.8, 0.9	0.9, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.010.001.000_mod_sub_strata_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	5	3
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.01	-0.01
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.03, 0.03)	0.00 (-0.05, 0.05)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.08, 0.08)
P-value ^b		0.9469

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.10.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.12 (-3.09, 3.31)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female Tanner Stage > I		
Baseline		
n	8	8
Mean (SD)	0.91 (0.06)	0.89 (0.05)
Median	0.92	0.90
25th, 75th Percentile	0.86, 0.96	0.86, 0.92
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	8	8
Mean (SD)	0.92 (0.07)	0.89 (0.05)
Median	0.91	0.89
25th, 75th Percentile	0.87, 0.95	0.86, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	8
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.00)	0.00 (0.00, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.01 (0.00, 0.01)
P-value ^b		0.1148

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.10.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Strata for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		1.02 (-0.24, 2.23)
P-value for interaction term, treatment * [Strata]		0.4580

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and stratum interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Baseline		
n	10	13
Mean (SD)	0.87 (0.05)	0.90 (0.03)
Median	0.88	0.90
25th, 75th Percentile	0.84, 0.92	0.88, 0.91
Min, Max	0.8, 0.9	0.8, 1.0
Week 52		
n	10	13
Mean (SD)	0.88 (0.04)	0.90 (0.03)
Median	0.88	0.90
25th, 75th Percentile	0.84, 0.91	0.89, 0.91
Min, Max	0.8, 0.9	0.8, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	10	13
Mean (SD)	0.00 (0.02)	0.00 (0.02)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.02)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.02, 0.01)
P-value ^b		0.7961

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.12 (-1.04, 0.80)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Baseline		
n	24	18
Mean (SD)	0.91 (0.04)	0.89 (0.04)
Median	0.91	0.89
25th, 75th Percentile	0.88, 0.94	0.86, 0.91
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	24	18
Mean (SD)	0.91 (0.05)	0.89 (0.04)
Median	0.91	0.88
25th, 75th Percentile	0.87, 0.94	0.86, 0.89
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	18
Mean (SD)	0.00 (0.02)	0.00 (0.02)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	-0.1, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.02, 0.01)
P-value ^b		0.5913

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.18 (-0.83, 0.47)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Baseline		
n	19	22
Mean (SD)	0.90 (0.04)	0.91 (0.08)
Median	0.90	0.91
25th, 75th Percentile	0.87, 0.92	0.86, 0.92
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	19	22
Mean (SD)	0.91 (0.04)	0.90 (0.04)
Median	0.90	0.91
25th, 75th Percentile	0.87, 0.93	0.87, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	22
Mean (SD)	0.00 (0.01)	-0.01 (0.07)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.03, 0.04)	-0.02 (-0.04, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.05, 0.02)
P-value ^b		0.2993

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.11.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.36 (-1.03, 0.32)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Baseline		
n	8	5
Mean (SD)	0.89 (0.05)	0.92 (0.05)
Median	0.90	0.92
25th, 75th Percentile	0.89, 0.91	0.91, 0.93
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	8	5
Mean (SD)	0.90 (0.04)	0.91 (0.05)
Median	0.90	0.92
25th, 75th Percentile	0.88, 0.91	0.89, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.011.001.000_mod_sub_bhgt_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.11.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	8	5
Mean (SD)	0.00 (0.02)	-0.01 (0.01)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.01 (-0.02, 0.04)	-0.01 (-0.03, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.07, 0.02)
P-value ^b		0.2266

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.011.001.000_mod_sub_bhgt_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.11.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline Height Z-score for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-1.45 (-3.62, 0.85)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.6110

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and height z-score interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.011.001.000_mod_sub_bhgt_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.12.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Baseline		
n	19	18
Mean (SD)	0.91 (0.05)	0.91 (0.04)
Median	0.91	0.90
25th, 75th Percentile	0.89, 0.94	0.88, 0.94
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	19	18
Mean (SD)	0.91 (0.05)	0.90 (0.04)
Median	0.92	0.90
25th, 75th Percentile	0.88, 0.94	0.88, 0.93
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.12.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	19	18
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.01)
P-value ^b		0.5142

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.12.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.25 (-0.97, 0.49)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.12.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Baseline		
n	18	14
Mean (SD)	0.89 (0.04)	0.89 (0.04)
Median	0.89	0.89
25th, 75th Percentile	0.87, 0.92	0.86, 0.91
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	18	14
Mean (SD)	0.89 (0.04)	0.89 (0.04)
Median	0.89	0.89
25th, 75th Percentile	0.87, 0.92	0.86, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.12.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	14
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.02)
P-value ^b		0.5754

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.12.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.22 (-0.55, 0.99)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.12.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Baseline		
n	24	26
Mean (SD)	0.89 (0.04)	0.90 (0.07)
Median	0.89	0.90
25th, 75th Percentile	0.87, 0.92	0.86, 0.92
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	24	26
Mean (SD)	0.90 (0.04)	0.89 (0.04)
Median	0.90	0.90
25th, 75th Percentile	0.87, 0.92	0.86, 0.92
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.12.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	24	26
Mean (SD)	0.01 (0.01)	-0.01 (0.06)
Median	0.01	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.01 (-0.02, 0.04)	-0.01 (-0.03, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.05, 0.01)
P-value ^b		0.2351

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.12.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Baseline AGV Category for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.40 (-1.06, 0.26)
P-value for interaction term, treatment * [Baseline AGV]		0.2403

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and AGV interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.13.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Baseline		
n	41	44
Mean (SD)	0.89 (0.05)	0.89 (0.03)
Median	0.89	0.89
25th, 75th Percentile	0.87, 0.92	0.87, 0.91
Min, Max	0.8, 1.0	0.8, 1.0
Week 52		
n	41	44
Mean (SD)	0.90 (0.05)	0.89 (0.03)
Median	0.89	0.89
25th, 75th Percentile	0.87, 0.92	0.87, 0.91
Min, Max	0.8, 1.0	0.8, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.13.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	41	44
Mean (SD)	0.00 (0.01)	0.00 (0.01)
Median	0.01	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.01
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (0.00, 0.01)	0.00 (-0.01, 0.00)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.00)
P-value ^b		0.1803

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

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Table 14.2.7.13.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.30 (-0.74, 0.14)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.13.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Baseline		
n	20	14
Mean (SD)	0.91 (0.04)	0.94 (0.09)
Median	0.92	0.92
25th, 75th Percentile	0.89, 0.94	0.90, 0.97
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	20	14
Mean (SD)	0.91 (0.04)	0.92 (0.05)
Median	0.92	0.92
25th, 75th Percentile	0.90, 0.93	0.89, 0.96
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.013.001.000_mod_sub_eth_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.13.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	20	14
Mean (SD)	0.00 (0.01)	-0.02 (0.08)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.04, 0.03)	-0.02 (-0.06, 0.02)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.06, 0.02)
P-value ^b		0.3345

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.013.001.000_mod_sub_eth_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.13.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Ethnicity for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.36 (-1.09, 0.37)
P-value for interaction term,treatment * [Ethnicity]		0.1463

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and ethnicity interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.013.001.000_mod_sub_eth_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
North America		
Baseline		
n	26	27
Mean (SD)	0.90 (0.05)	0.91 (0.08)
Median	0.89	0.90
25th, 75th Percentile	0.86, 0.93	0.86, 0.95
Min, Max	0.8, 1.0	0.8, 1.2
Week 52		
n	26	27
Mean (SD)	0.90 (0.04)	0.90 (0.04)
Median	0.90	0.89
25th, 75th Percentile	0.87, 0.94	0.86, 0.93
Min, Max	0.8, 1.0	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	26	27
Mean (SD)	0.01 (0.01)	-0.01 (0.06)
Median	0.01	0.00
25th, 75th Percentile	0.00, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	-0.3, 0.0
LS mean change from baseline (95% CI)	0.01 (-0.02, 0.03)	-0.01 (-0.03, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		-0.02 (-0.04, 0.01)
P-value ^b		0.1962

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		-0.39 (-0.98, 0.20)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Europe		
Baseline		
n	18	17
Mean (SD)	0.91 (0.05)	0.89 (0.03)
Median	0.90	0.90
25th, 75th Percentile	0.89, 0.94	0.88, 0.91
Min, Max	0.8, 1.0	0.8, 0.9
Week 52		
n	18	17
Mean (SD)	0.91 (0.05)	0.89 (0.03)
Median	0.91	0.90
25th, 75th Percentile	0.89, 0.92	0.87, 0.91
Min, Max	0.8, 1.0	0.8, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	18	17
Mean (SD)	0.00 (0.02)	0.00 (0.02)
Median	0.00	0.00
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.02
Min, Max	0.0, 0.0	-0.1, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.01)
P-value ^b		0.9570

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
 Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
 Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.02 (-0.65, 0.69)

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Japan		
Baseline		
n	4	2
Mean (SD)	0.89 (0.03)	0.91 (0.01)
Median	0.89	0.91
25th, 75th Percentile	0.87, 0.91	0.90, 0.91
Min, Max	0.9, 0.9	0.9, 0.9
Week 52		
n	4	2
Mean (SD)	0.89 (0.03)	0.90 (0.01)
Median	0.90	0.90
25th, 75th Percentile	0.87, 0.91	0.89, 0.91
Min, Max	0.8, 0.9	0.9, 0.9

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	4	2
Mean (SD)	0.00 (0.01)	-0.01 (0.01)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.01	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	NE	NE
Difference in LS mean change from baseline (95% CI) ^a		NE
P-value ^b		NE
SMD (95% CI) ^c		NE

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
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NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Rest of World		
Baseline		
n	13	12
Mean (SD)	0.89 (0.03)	0.89 (0.04)
Median	0.90	0.90
25th, 75th Percentile	0.87, 0.92	0.88, 0.91
Min, Max	0.8, 0.9	0.8, 0.9
Week 52		
n	13	12
Mean (SD)	0.89 (0.03)	0.89 (0.04)
Median	0.90	0.89
25th, 75th Percentile	0.87, 0.92	0.86, 0.91
Min, Max	0.8, 0.9	0.8, 1.0

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

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Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Change from baseline		
n	13	12
Mean (SD)	0.00 (0.01)	0.00 (0.02)
Median	0.00	-0.01
25th, 75th Percentile	-0.01, 0.00	-0.01, 0.00
Min, Max	0.0, 0.0	0.0, 0.0
LS mean change from baseline (95% CI)	0.00 (-0.01, 0.01)	0.00 (-0.01, 0.01)
Difference in LS mean change from baseline (95% CI) ^a		0.00 (-0.01, 0.02)
P-value ^b		0.5708

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.7.14.1
Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 by Region for BMN111-301
Analysis Population: Full Analysis Set

Arm Span to Standing Height Ratio	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
SMD (95% CI) ^c		0.25 (-0.61, 1.11)
P-value for interaction term, treatment * [Region]		0.4345

NE, Not estimable.

^a Difference is 15ug/kg BMN 111 minus placebo. ^b Two-sided p-value.

^c SMD (standardized mean difference) is an effect size measure similar to hedges g; however, this SMD is based on a least squares mean from a general linear model. The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

LS means and difference in LS means were obtained from an analysis of covariance model. For the by-stratum analysis, model terms included stratum defined by sex and Tanner stage, treatment, baseline age, baseline AGV, baseline height z-score, and baseline arm span to standing height ratio.

For the overall analysis, which includes the interaction-term, model terms include baseline age, baseline AGV, baseline height z-score, baseline arm span to standing height ratio, and treatment and region interaction.

Missing assessments at week 52 were not imputed.

Report: mi897809 02AUG2023 01:20 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.014.001.000_mod_sub_reg_armsphgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_hedge2_sub_301.sas, Database: N/A

Table 14.2.10.3.2
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Arm Length to Lower Arm (Forearm) Length Ratio at Week 52 for
BMN111-301
Analysis Population: Full Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5226
Baseline Age Group*Treatment Interaction	0.2776
Baseline Tanner Stage*Treatment Interaction	0.7417
Strata*Treatment Interaction	0.3813
Baseline Height Z-Score Category*Treatment Interaction	0.6082
Baseline AGV Category*Treatment Interaction	0.5864
Ethnicity*Treatment Interaction	0.0261
Region*Treatment Interaction	0.4558

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 02AUG2023 01:58 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.003.002.000_mod_armrt_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval2_sub_301.sas, Database: N/A

Table 14.2.10.3.3

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Leg Length (Thigh) to Knee to Heel Length Ratio at Week 52 for BMN111-301
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3075
Baseline Age Group*Treatment Interaction	0.7255
Baseline Tanner Stage*Treatment Interaction	0.5515
Strata*Treatment Interaction	0.6307
Baseline Height Z-Score Category*Treatment Interaction	0.7456
Baseline AGV Category*Treatment Interaction	0.6958
Ethnicity*Treatment Interaction	0.7392
Region*Treatment Interaction	0.7366

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 02AUG2023 01:58 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.003.003.000_mod_legrt_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval2_sub_301.sas, Database: N/A

Table 14.2.10.3.4
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Upper Leg Length (Thigh) to Tibial Length Ratio at Week 52 for BMN111-301
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9360
Baseline Age Group*Treatment Interaction	0.7615
Baseline Tanner Stage*Treatment Interaction	0.0719
Strata*Treatment Interaction	0.2941
Baseline Height Z-Score Category*Treatment Interaction	0.3131
Baseline AGV Category*Treatment Interaction	0.7825
Ethnicity*Treatment Interaction	0.8246
Region*Treatment Interaction	0.9665

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 02AUG2023 01:58 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.003.004.000_mod_legtrt_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval2_sub_301.sas, Database: N/A

Table 14.2.10.3.5
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Arm Span to Standing Height Ratio at Week 52 for BMN111-301
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.2137
Baseline Age Group*Treatment Interaction	0.8808
Baseline Tanner Stage*Treatment Interaction	0.5821
Strata*Treatment Interaction	0.4580
Baseline Height Z-Score Category*Treatment Interaction	0.6110
Baseline AGV Category*Treatment Interaction	0.2403
Ethnicity*Treatment Interaction	0.1463
Region*Treatment Interaction	0.4345

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 02AUG2023 01:58 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.003.005.000_mod_armsphgt_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_mod_int_pval2_sub_301.sas, Database: N/A

Table 14.2.7.1.1.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	32	30
Mean (SD)	73.00 (16.36)	73.33 (18.18)
Median	75.55	77.18
25th, 75th Percentile	62.50, 85.33	56.52, 88.04
Min, Max	30.4, 100.0	30.4, 100.0
Week 26		
n	31	27
Mean (SD)	74.48 (16.84)	69.98 (18.00)
Median	77.17	70.65

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.001.001_qs_sum_ovr_ped_care_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.48, 88.04	56.52, 81.52
Min, Max	30.4, 97.8	26.1, 97.8
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	1.02 (12.18)	-3.36 (20.04)
Median	0.00	-1.09
25th, 75th Percentile	-7.61, 8.60	-6.53, 6.53
Min, Max	-28.3, 30.4	-70.7, 26.4
Week 52		
n	32	29
Mean (SD)	72.73 (16.06)	73.64 (18.27)
Median	72.83	78.26

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.1.1.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 88.59	66.30, 89.13
Min, Max	39.1, 96.7	25.0, 94.6
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	-0.77 (14.26)	0.63 (21.16)
Median	2.96	2.17
25th, 75th Percentile	-6.53, 9.09	-7.61, 11.96
Min, Max	-38.0, 21.7	-75.0, 38.1
Difference in change from baseline (95%CI)		1.40 (-8.03, 10.83)
P-value ^b		0.7668
Hedges'g (95% CI) ^c		0.08 (-0.43, 0.58)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	27	29
Mean (SD)	70.89 (17.53)	69.51 (13.89)
Median	70.65	68.48
25th, 75th Percentile	57.61, 84.78	59.78, 79.35
Min, Max	33.0, 98.9	45.7, 96.7
Week 26		
n	28	27
Mean (SD)	73.54 (17.08)	74.26 (15.07)
Median	76.63	73.91

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.70, 87.82	66.30, 86.96
Min, Max	39.1, 96.7	40.2, 97.8
Change from baseline to Week 26 ^a		
n	27	27
Mean (SD)	3.24 (9.22)	4.35 (13.63)
Median	-1.09	5.43
25th, 75th Percentile	-4.35, 9.78	-6.52, 13.05
Min, Max	-8.1, 25.0	-23.9, 29.4
Week 52		
n	27	27
Mean (SD)	73.96 (17.02)	69.08 (12.97)
Median	80.43	64.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.1.1.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.87, 88.04	58.70, 76.09
Min, Max	35.9, 98.9	51.1, 96.7
Change from baseline to Week 52 ^a		
n	26	27
Mean (SD)	3.32 (10.49)	-0.83 (12.20)
Median	3.09	-1.09
25th, 75th Percentile	-3.26, 10.87	-7.61, 5.44
Min, Max	-27.2, 19.6	-28.3, 21.7
Difference in change from baseline (95%CI)		-4.15 (-10.43, 2.14)
P-value ^b		0.1912
Hedges'g (95% CI) ^c		-0.36 (-0.90, 0.19)
P-value for interaction term, treatment * [Sex]		0.3362

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	23	30
Mean (SD)	70.94 (16.76)	71.63 (16.82)
Median	70.65	72.83
25th, 75th Percentile	57.61, 85.87	60.87, 84.78
Min, Max	30.4, 97.8	30.4, 100.0
Week 26		
n	24	29
Mean (SD)	69.17 (18.45)	70.80 (19.19)
Median	73.92	70.65

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.26, 83.16	61.96, 85.87
Min, Max	30.4, 95.7	26.1, 97.8
Change from baseline to Week 26 ^a		
n	23	29
Mean (SD)	-1.92 (10.66)	-0.37 (17.05)
Median	-4.35	0.00
25th, 75th Percentile	-8.06, 7.61	-6.52, 8.70
Min, Max	-28.3, 16.3	-70.7, 22.8
Week 52		
n	22	30
Mean (SD)	72.63 (15.29)	69.89 (17.36)
Median	71.74	70.66

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.87, 85.87	58.70, 81.52
Min, Max	43.5, 98.9	25.0, 96.7
Change from baseline to Week 52 ^a		
n	21	30
Mean (SD)	0.41 (12.68)	-1.73 (18.00)
Median	1.08	-0.54
25th, 75th Percentile	-5.44, 9.78	-8.69, 8.70
Min, Max	-27.2, 19.6	-75.0, 21.7
Difference in change from baseline (95%CI)		-2.15 (-11.32, 7.03)
P-value ^b		0.6404
Hedges'g (95% CI) ^c		-0.13 (-0.69, 0.43)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	24	17
Mean (SD)	71.23 (18.82)	67.18 (15.39)
Median	72.80	63.04
25th, 75th Percentile	56.52, 86.42	55.43, 80.43
Min, Max	33.0, 100.0	42.1, 92.4
Week 26		
n	23	15
Mean (SD)	75.26 (15.65)	71.23 (11.79)
Median	77.17	69.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	64.77, 88.04	66.30, 81.52
Min, Max	39.1, 93.5	48.9, 92.4
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	3.25 (10.61)	3.65 (16.65)
Median	0.00	-1.08
25th, 75th Percentile	-3.86, 10.87	-6.52, 20.65
Min, Max	-13.1, 30.4	-23.9, 29.4
Week 52		
n	24	15
Mean (SD)	71.71 (17.38)	70.92 (13.20)
Median	70.11	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.79, 89.68	60.87, 82.61
Min, Max	35.9, 94.6	43.2, 89.1
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	0.48 (11.89)	3.34 (16.18)
Median	3.11	1.09
25th, 75th Percentile	-5.64, 5.98	-8.69, 19.57
Min, Max	-29.4, 21.7	-28.3, 32.6
Difference in change from baseline (95%CI)		2.86 (-6.25, 11.98)
P-value ^b		0.5284
Hedges'g (95% CI) ^c		0.21 (-0.44, 0.85)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	12	12
Mean (SD)	75.72 (12.86)	77.08 (15.11)
Median	79.35	80.44
25th, 75th Percentile	65.76, 83.16	61.42, 90.76
Min, Max	50.0, 94.6	52.2, 97.8
Week 26		
n	12	10
Mean (SD)	81.39 (13.24)	77.28 (14.87)
Median	79.89	82.07

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	72.24, 93.48	67.39, 90.22
Min, Max	57.6, 97.8	48.9, 92.4
Change from baseline to Week 26 ^a		
n	11	10
Mean (SD)	7.95 (9.11)	-1.74 (20.64)
Median	7.60	1.64
25th, 75th Percentile	-1.09, 11.96	-3.26, 13.04
Min, Max	-3.3, 25.0	-48.9, 20.7
Week 52		
n	13	11
Mean (SD)	77.34 (16.86)	76.38 (15.76)
Median	82.61	79.35

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.001.002_qs_sum_ovr_ped_care_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	72.83, 91.30	59.78, 90.22
Min, Max	39.1, 96.7	53.3, 93.5
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	3.53 (15.17)	-0.20 (17.67)
Median	9.24	-2.18
25th, 75th Percentile	0.55, 13.59	-6.52, 4.35
Min, Max	-38.0, 15.2	-37.0, 38.1
Difference in change from baseline (95%CI)		-3.73 (-17.97, 10.51)
P-value ^b		0.5918
Hedges'g (95% CI) ^c		-0.22 (-1.04, 0.60)
P-value for interaction term, treatment * [Age at Baseline]		0.6611

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	46	47
Mean (SD)	73.19 (16.46)	70.73 (16.35)
Median	75.55	71.74
25th, 75th Percentile	60.87, 85.87	56.52, 84.78
Min, Max	30.4, 100.0	30.4, 100.0
Week 26		
n	46	43
Mean (SD)	73.72 (16.87)	70.83 (17.06)
Median	78.26	70.65

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.96, 86.96	64.13, 83.70
Min, Max	30.4, 95.7	26.1, 97.8
Change from baseline to Week 26 ^a		
n	44	43
Mean (SD)	0.55 (11.07)	0.37 (16.32)
Median	-1.09	0.00
25th, 75th Percentile	-7.61, 9.09	-6.52, 11.95
Min, Max	-28.3, 30.4	-70.7, 26.4
Week 52		
n	46	45
Mean (SD)	73.59 (16.67)	70.62 (16.63)
Median	71.74	71.74

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.87, 89.13	58.70, 82.61
Min, Max	39.1, 98.9	25.0, 96.7
Change from baseline to Week 52 ^a		
n	44	45
Mean (SD)	0.14 (12.98)	0.25 (18.89)
Median	2.17	2.17
25th, 75th Percentile	-5.65, 7.81	-8.69, 9.78
Min, Max	-38.0, 21.7	-75.0, 38.1
Difference in change from baseline (95%CI)		0.10 (-6.72, 6.93)
P-value ^b		0.9757
Hedges'g (95% CI) ^c		0.01 (-0.41, 0.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	13	12
Mean (SD)	67.92 (17.97)	74.28 (15.90)
Median	67.39	71.20
25th, 75th Percentile	59.78, 82.61	60.87, 90.76
Min, Max	33.0, 92.4	53.3, 97.8
Week 26		
n	13	11
Mean (SD)	75.13 (17.27)	77.17 (14.12)
Median	76.09	80.43

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	69.57, 90.22	66.30, 90.22
Min, Max	39.1, 97.8	48.9, 92.4
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	7.21 (8.51)	0.99 (22.10)
Median	6.53	1.09
25th, 75th Percentile	1.73, 9.78	-3.26, 20.65
Min, Max	-3.3, 25.0	-48.9, 29.4
Week 52		
n	13	11
Mean (SD)	72.24 (15.89)	74.80 (12.96)
Median	73.91	76.09

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.22, 82.61	61.96, 89.13
Min, Max	35.9, 91.3	54.4, 90.2
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	4.32 (11.78)	-1.38 (8.45)
Median	7.60	-2.18
25th, 75th Percentile	2.92, 11.96	-6.52, 1.09
Min, Max	-27.2, 15.2	-14.1, 19.6
Difference in change from baseline (95%CI)		-5.70 (-14.54, 3.13)
P-value ^b		0.1942
Hedges'g (95% CI) ^c		-0.53 (-1.34, 0.29)
P-value for interaction term, treatment *[Baseline Tanner Stage]		0.4105

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	10	15
Mean (SD)	64.95 (16.18)	66.97 (14.99)
Median	60.63	61.36
25th, 75th Percentile	51.09, 84.78	53.26, 82.61
Min, Max	50.0, 90.2	42.1, 90.2
Week 26		
n	10	11
Mean (SD)	64.43 (19.80)	70.55 (10.63)
Median	60.87	70.65

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 77.17	67.39, 76.09
Min, Max	37.0, 95.7	44.6, 85.9
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-0.53 (14.09)	3.15 (16.70)
Median	-2.48	8.70
25th, 75th Percentile	-5.43, 9.78	-14.13, 13.05
Min, Max	-28.3, 21.7	-23.9, 26.4
Week 52		
n	9	12
Mean (SD)	68.23 (17.41)	65.85 (11.65)
Median	65.91	64.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.87, 80.43	56.52, 76.09
Min, Max	43.5, 93.2	51.1, 90.2
Change from baseline to Week 52 ^a		
n	9	12
Mean (SD)	2.47 (12.48)	-0.28 (17.45)
Median	2.96	-1.09
25th, 75th Percentile	-5.44, 10.87	-10.87, 3.56
Min, Max	-21.7, 19.6	-28.3, 38.1
Difference in change from baseline (95%CI)		-2.75 (-17.11, 11.61)
P-value ^b		0.6930
Hedges'g (95% CI) ^c		-0.17 (-1.03, 0.70)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	23	17
Mean (SD)	78.07 (14.14)	67.28 (16.09)
Median	81.52	61.96
25th, 75th Percentile	64.13, 89.13	55.43, 75.00
Min, Max	53.3, 100.0	45.7, 100.0
Week 26		
n	22	17
Mean (SD)	78.46 (12.37)	65.18 (16.79)
Median	79.89	68.48

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	72.73, 89.13	52.17, 79.35
Min, Max	47.8, 93.5	29.4, 92.4
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	-0.57 (7.21)	-2.10 (21.78)
Median	-2.17	-5.43
25th, 75th Percentile	-5.44, 4.35	-6.52, 14.29
Min, Max	-13.0, 12.0	-70.7, 23.9
Week 52		
n	23	17
Mean (SD)	76.51 (16.03)	64.63 (17.26)
Median	81.52	64.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.22, 89.13	59.78, 79.35
Min, Max	39.1, 98.9	25.0, 89.1
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-2.32 (14.16)	-2.66 (22.31)
Median	1.09	1.09
25th, 75th Percentile	-5.43, 7.60	-8.69, 5.44
Min, Max	-38.0, 15.2	-75.0, 32.6
Difference in change from baseline (95%CI)		-0.33 (-12.20, 11.54)
P-value ^b		0.9550
Hedges'g (95% CI) ^c		-0.02 (-0.65, 0.62)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	18	22
Mean (SD)	66.38 (18.34)	75.90 (16.90)
Median	70.11	79.89
25th, 75th Percentile	58.70, 78.26	63.04, 85.87
Min, Max	30.4, 98.9	30.4, 97.8
Week 26		
n	19	21
Mean (SD)	71.26 (17.98)	76.63 (18.16)
Median	76.09	80.43

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	57.61, 83.70	66.30, 89.77
Min, Max	30.4, 96.7	26.1, 97.8
Change from baseline to Week 26 ^a		
n	18	21
Mean (SD)	5.64 (12.48)	1.16 (14.13)
Median	5.27	0.00
25th, 75th Percentile	-7.61, 14.18	-3.26, 6.53
Min, Max	-16.3, 30.4	-48.9, 22.8
Week 52		
n	19	22
Mean (SD)	70.56 (16.99)	79.25 (14.45)
Median	70.65	81.52

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.52, 88.04	75.00, 90.22
Min, Max	35.9, 94.6	45.7, 96.7
Change from baseline to Week 52 ^a		
n	18	22
Mean (SD)	5.08 (9.86)	3.35 (11.82)
Median	4.89	3.27
25th, 75th Percentile	1.08, 11.96	-3.26, 11.96
Min, Max	-15.2, 19.6	-26.2, 21.7
Difference in change from baseline (95%CI)		-1.72 (-8.79, 5.35)
P-value ^b		0.6249
Hedges'g (95% CI) ^c		-0.15 (-0.78, 0.47)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	8	5
Mean (SD)	76.22 (16.12)	79.56 (11.79)
Median	79.90	79.35
25th, 75th Percentile	66.85, 89.13	73.91, 90.22
Min, Max	45.7, 92.4	63.0, 91.3
Week 26		
n	8	5
Mean (SD)	80.44 (16.94)	80.22 (13.61)
Median	85.87	84.78

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	72.29, 91.85	67.39, 92.39
Min, Max	45.7, 97.8	64.1, 92.4
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	4.21 (9.52)	0.65 (19.41)
Median	3.26	1.09
25th, 75th Percentile	-1.09, 11.42	-9.78, 5.43
Min, Max	-10.9, 17.4	-22.8, 29.4
Week 52		
n	8	5
Mean (SD)	76.22 (15.10)	73.70 (14.41)
Median	75.00	76.09

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	66.85, 90.76	66.30, 82.61
Min, Max	51.1, 93.5	53.3, 90.2
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	0.00 (14.20)	-5.87 (20.29)
Median	2.72	-3.26
25th, 75th Percentile	-6.53, 6.52	-7.61, -1.08
Min, Max	-27.2, 21.7	-37.0, 19.6
Difference in change from baseline (95%CI)		-5.87 (-26.79, 15.05)
P-value ^b		0.5495
Hedges'g (95% CI) ^c		-0.33 (-1.45, 0.81)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.9547

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	19	19
Mean (SD)	68.61 (17.49)	76.03 (14.24)
Median	65.22	77.17
25th, 75th Percentile	57.61, 83.70	63.04, 88.04
Min, Max	33.0, 100.0	52.2, 100.0
Week 26		
n	19	17
Mean (SD)	70.53 (16.71)	70.59 (19.37)
Median	72.73	70.65

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.96, 83.70	56.52, 83.70
Min, Max	37.0, 91.3	29.4, 97.8
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	1.92 (11.77)	-4.54 (20.87)
Median	6.18	-4.35
25th, 75th Percentile	-7.61, 9.79	-10.87, 6.53
Min, Max	-28.3, 21.7	-70.7, 22.8
Week 52		
n	18	18
Mean (SD)	72.75 (18.50)	70.59 (19.41)
Median	73.37	69.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.22, 90.22	55.43, 89.13
Min, Max	35.9, 94.6	25.0, 96.7
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	3.53 (12.71)	-5.07 (21.29)
Median	7.06	0.01
25th, 75th Percentile	-3.26, 10.87	-8.69, 5.44
Min, Max	-29.4, 19.6	-75.0, 21.7
Difference in change from baseline (95%CI)		-8.60 (-20.57, 3.38)
P-value ^b		0.1526
Hedges'g (95% CI) ^c		-0.48 (-1.14, 0.19)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	18	13
Mean (SD)	72.52 (16.99)	67.97 (18.72)
Median	77.72	70.65
25th, 75th Percentile	63.04, 82.61	55.43, 84.78
Min, Max	30.4, 94.6	30.4, 92.4
Week 26		
n	16	11
Mean (SD)	79.22 (18.28)	71.84 (19.84)
Median	83.15	73.91

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.43, 93.48	66.30, 88.04
Min, Max	30.4, 97.8	26.1, 92.4
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	5.79 (10.61)	3.07 (15.53)
Median	0.87	-2.17
25th, 75th Percentile	-1.63, 10.87	-5.43, 20.66
Min, Max	-6.5, 30.4	-18.5, 29.4
Week 52		
n	18	12
Mean (SD)	74.52 (16.80)	73.71 (16.96)
Median	77.17	80.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.96, 88.04	60.87, 88.04
Min, Max	39.1, 96.7	43.2, 90.2
Change from baseline to Week 52 ^a		
n	18	12
Mean (SD)	1.99 (15.29)	6.32 (19.03)
Median	3.81	5.44
25th, 75th Percentile	-3.26, 11.96	-5.98, 17.40
Min, Max	-38.0, 21.7	-28.3, 38.1
Difference in change from baseline (95%CI)		4.32 (-8.55, 17.19)
P-value ^b		0.4969
Hedges'g (95% CI) ^c		0.25 (-0.49, 0.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	22	27
Mean (SD)	74.58 (16.28)	69.91 (16.09)
Median	77.18	65.22
25th, 75th Percentile	63.64, 86.96	58.70, 80.43
Min, Max	45.7, 98.9	42.1, 97.8
Week 26		
n	24	26
Mean (SD)	73.34 (15.72)	73.24 (13.51)
Median	77.72	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.70, 84.79	66.30, 82.61
Min, Max	44.6, 93.5	40.2, 93.5
Change from baseline to Week 26 ^a		
n	22	26
Mean (SD)	-0.51 (9.85)	2.69 (15.58)
Median	-3.57	2.72
25th, 75th Percentile	-7.61, 4.35	-3.26, 13.04
Min, Max	-16.3, 17.4	-48.9, 26.4
Week 52		
n	23	26
Mean (SD)	72.76 (14.92)	70.99 (13.20)
Median	70.65	73.92

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.87, 88.04	61.96, 78.26
Min, Max	48.9, 98.9	40.2, 93.5
Change from baseline to Week 52 ^a		
n	21	26
Mean (SD)	-1.76 (10.19)	0.44 (12.33)
Median	1.08	-1.63
25th, 75th Percentile	-6.52, 4.34	-7.61, 4.35
Min, Max	-27.2, 19.6	-26.2, 24.3
Difference in change from baseline (95%CI)		2.19 (-4.56, 8.95)
P-value ^b		0.5163
Hedges'g (95% CI) ^c		0.19 (-0.39, 0.76)
P-value for interaction term, treatment * [Baseline AGV]		0.1622

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	40	44
Mean (SD)	71.57 (15.63)	71.06 (15.08)
Median	71.20	71.20
25th, 75th Percentile	60.87, 83.16	60.33, 84.78
Min, Max	33.0, 100.0	30.4, 97.8
Week 26		
n	40	41
Mean (SD)	74.52 (15.99)	72.85 (15.17)
Median	78.26	73.91

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	64.77, 88.04	66.30, 83.70
Min, Max	37.0, 95.7	26.1, 97.8
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	3.53 (11.51)	1.95 (12.08)
Median	4.35	0.00
25th, 75th Percentile	-4.35, 10.87	-6.52, 11.95
Min, Max	-28.3, 30.4	-22.8, 23.9
Week 52		
n	39	43
Mean (SD)	72.51 (17.23)	72.21 (14.39)
Median	71.74	76.09

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.96, 88.04	59.78, 83.70
Min, Max	35.9, 98.9	43.2, 96.7
Change from baseline to Week 52 ^a		
n	38	43
Mean (SD)	0.68 (13.63)	1.42 (13.97)
Median	3.63	-1.08
25th, 75th Percentile	-5.44, 9.78	-7.61, 8.70
Min, Max	-38.0, 21.7	-37.0, 38.1
Difference in change from baseline (95%CI)		0.74 (-5.38, 6.86)
P-value ^b		0.8092
Hedges'g (95% CI) ^c		0.05 (-0.38, 0.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.001.007_qs_sum_ovr_ped_care_tot_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.1.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported PedsQL : Total Score		
Baseline		
n	19	15
Mean (SD)	73.00 (19.44)	72.62 (19.63)
Median	78.26	71.74
25th, 75th Percentile	53.26, 89.13	53.57, 92.39
Min, Max	30.4, 98.9	42.1, 100.0
Week 26		
n	19	13
Mean (SD)	72.99 (18.85)	69.82 (20.96)
Median	76.09	68.48

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.1.1.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.35, 86.96	66.30, 89.77
Min, Max	30.4, 97.8	29.4, 97.8
Change from baseline to Week 26 ^a		
n	18	13
Mean (SD)	-1.09 (8.72)	-4.10 (28.70)
Median	-2.17	4.35
25th, 75th Percentile	-7.61, 2.17	-5.43, 11.95
Min, Max	-13.1, 25.0	-70.7, 29.4
Week 52		
n	20	13
Mean (SD)	74.82 (14.86)	68.90 (20.82)
Median	77.72	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.1.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.96, 88.59	58.70, 82.61
Min, Max	48.9, 93.2	25.0, 94.6
Change from baseline to Week 52 ^a		
n	19	13
Mean (SD)	1.93 (11.04)	-5.02 (25.54)
Median	2.96	0.00
25th, 75th Percentile	-3.26, 10.87	-13.35, 4.35
Min, Max	-27.2, 19.6	-75.0, 24.3
Difference in change from baseline (95%CI)		-6.95 (-22.98, 9.07)
P-value ^b		0.3699
Hedges'g (95% CI) ^c		-0.37 (-1.08, 0.34)
P-value for interaction term, treatment * [Ethnicity]		0.2332

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	32	30
Mean (SD)	73.34 (19.68)	72.93 (26.78)
Median	75.00	85.94
25th, 75th Percentile	59.38, 89.07	53.13, 93.75
Min, Max	28.1, 100.0	3.6, 100.0
Week 26		
n	31	27
Mean (SD)	73.46 (20.17)	68.32 (23.97)
Median	75.00	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.1.2.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 90.63	50.00, 87.50
Min, Max	18.8, 100.0	15.6, 100.0
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	0.28 (18.43)	-5.08 (24.53)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 9.37	-12.50, 6.25
Min, Max	-62.5, 40.6	-81.3, 46.4
Week 52		
n	32	29
Mean (SD)	71.19 (18.75)	73.14 (26.55)
Median	71.88	87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 85.94	46.88, 93.75
Min, Max	34.4, 100.0	9.4, 100.0
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	-1.71 (19.78)	0.92 (26.93)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 9.38	-9.37, 9.38
Min, Max	-65.6, 37.5	-90.6, 65.6
Difference in change from baseline (95%CI)		2.64 (-9.52, 14.79)
P-value ^b		0.6657
Hedges'g (95% CI) ^c		0.11 (-0.40, 0.62)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	27	29
Mean (SD)	67.69 (21.93)	68.49 (20.28)
Median	75.00	68.75
25th, 75th Percentile	46.88, 81.25	53.13, 87.50
Min, Max	25.0, 100.0	21.9, 100.0
Week 26		
n	28	27
Mean (SD)	72.10 (24.72)	73.20 (21.46)
Median	79.69	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 90.63	59.38, 93.75
Min, Max	25.0, 100.0	28.1, 100.0
Change from baseline to Week 26 ^a		
n	27	27
Mean (SD)	4.76 (17.04)	5.08 (15.96)
Median	3.12	3.12
25th, 75th Percentile	0.00, 18.75	-6.25, 13.84
Min, Max	-40.6, 37.5	-18.8, 43.8
Week 52		
n	27	27
Mean (SD)	71.51 (19.37)	65.86 (19.69)
Median	78.13	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.1.2.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 87.50	50.00, 75.00
Min, Max	28.1, 96.9	31.3, 100.0
Change from baseline to Week 52 ^a		
n	26	27
Mean (SD)	4.69 (12.72)	-2.27 (19.08)
Median	3.13	0.00
25th, 75th Percentile	-3.13, 12.50	-18.75, 9.38
Min, Max	-25.0, 25.0	-59.4, 28.1
Difference in change from baseline (95%CI)		-6.95 (-15.89, 1.99)
P-value ^b		0.1243
Hedges'g (95% CI) ^c		-0.42 (-0.96, 0.13)
P-value for interaction term, treatment * [Sex]		0.2162

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	23	30
Mean (SD)	69.70 (19.39)	69.96 (23.40)
Median	75.00	73.44
25th, 75th Percentile	59.38, 81.25	50.00, 90.63
Min, Max	28.1, 100.0	15.6, 100.0
Week 26		
n	24	29
Mean (SD)	64.19 (23.26)	67.75 (25.61)
Median	67.19	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 82.82	46.88, 90.63
Min, Max	18.8, 100.0	15.6, 100.0
Change from baseline to Week 26 ^a		
n	23	29
Mean (SD)	-5.03 (19.80)	-1.71 (18.52)
Median	0.00	0.00
25th, 75th Percentile	-15.62, 9.37	-6.25, 6.25
Min, Max	-62.5, 18.8	-81.3, 25.0
Week 52		
n	22	30
Mean (SD)	68.90 (15.80)	66.04 (25.01)
Median	67.19	67.19

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 81.25	50.00, 87.50
Min, Max	37.5, 96.9	9.4, 100.0
Change from baseline to Week 52 ^a		
n	21	30
Mean (SD)	0.00 (17.20)	-3.91 (24.19)
Median	-3.12	0.00
25th, 75th Percentile	-9.37, 6.25	-9.38, 9.38
Min, Max	-28.1, 37.5	-90.6, 28.1
Difference in change from baseline (95%CI)		-3.92 (-16.27, 8.44)
P-value ^b		0.5273
Hedges'g (95% CI) ^c		-0.18 (-0.74, 0.38)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	24	17
Mean (SD)	66.91 (23.11)	67.12 (25.10)
Median	70.32	59.38
25th, 75th Percentile	46.88, 84.38	53.13, 90.63
Min, Max	25.0, 100.0	3.6, 100.0
Week 26		
n	23	15
Mean (SD)	74.73 (21.44)	71.46 (17.07)
Median	81.25	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 90.63	62.50, 81.25
Min, Max	25.0, 100.0	37.5, 93.8
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	6.27 (15.94)	5.18 (22.25)
Median	0.00	3.13
25th, 75th Percentile	-3.13, 18.75	-12.50, 21.87
Min, Max	-21.9, 40.6	-31.3, 46.4
Week 52		
n	24	15
Mean (SD)	69.78 (20.99)	69.73 (19.66)
Median	73.44	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.01, 85.94	46.88, 87.50
Min, Max	28.1, 100.0	40.6, 100.0
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	2.87 (13.48)	3.45 (21.16)
Median	1.57	0.00
25th, 75th Percentile	-3.13, 10.94	-13.39, 15.63
Min, Max	-25.0, 21.9	-25.0, 40.6
Difference in change from baseline (95%CI)		0.59 (-10.62, 11.79)
P-value ^b		0.9158
Hedges'g (95% CI) ^c		0.03 (-0.61, 0.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	12	12
Mean (SD)	80.47 (16.27)	77.87 (22.94)
Median	82.82	89.07
25th, 75th Percentile	67.19, 93.76	59.38, 95.32
Min, Max	46.9, 100.0	28.1, 100.0
Week 26		
n	12	10
Mean (SD)	86.39 (13.82)	78.44 (20.86)
Median	90.63	87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	73.44, 100.00	62.50, 93.75
Min, Max	62.5, 100.0	34.4, 96.9
Change from baseline to Week 26 ^a		
n	11	10
Mean (SD)	9.86 (11.15)	-2.81 (26.98)
Median	6.25	-1.57
25th, 75th Percentile	3.12, 18.75	-6.25, 6.25
Min, Max	-9.4, 31.3	-65.6, 43.8
Week 52		
n	13	11
Mean (SD)	78.37 (19.24)	79.26 (23.64)
Median	81.25	93.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 93.75	59.38, 96.88
Min, Max	34.4, 100.0	40.6, 100.0
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	0.00 (23.65)	2.84 (24.42)
Median	1.56	0.00
25th, 75th Percentile	-1.56, 10.94	-6.25, 6.25
Min, Max	-65.6, 25.0	-34.4, 65.6
Difference in change from baseline (95%CI)		2.84 (-18.01, 23.69)
P-value ^b		0.7797
Hedges'g (95% CI) ^c		0.11 (-0.71, 0.93)
P-value for interaction term, treatment * [Age at Baseline]		0.7788

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	46	47
Mean (SD)	71.19 (20.27)	69.26 (24.42)
Median	75.00	75.00
25th, 75th Percentile	59.38, 84.38	50.00, 90.63
Min, Max	28.1, 100.0	3.6, 100.0
Week 26		
n	46	43
Mean (SD)	71.40 (22.10)	68.51 (23.10)
Median	75.00	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 90.63	50.00, 87.50
Min, Max	18.8, 100.0	15.6, 100.0
Change from baseline to Week 26 ^a		
n	44	43
Mean (SD)	0.58 (19.04)	-0.65 (18.51)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 14.06	-9.37, 9.38
Min, Max	-62.5, 40.6	-81.3, 46.4
Week 52		
n	46	45
Mean (SD)	70.92 (18.98)	68.52 (24.33)
Median	71.88	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 87.50	50.00, 90.63
Min, Max	34.4, 100.0	9.4, 100.0
Change from baseline to Week 52 ^a		
n	44	45
Mean (SD)	0.50 (18.05)	-0.07 (25.50)
Median	0.00	0.00
25th, 75th Percentile	-7.81, 10.94	-9.38, 12.50
Min, Max	-65.6, 37.5	-90.6, 65.6
Difference in change from baseline (95%CI)		-0.57 (-9.87, 8.74)
P-value ^b		0.9037
Hedges'g (95% CI) ^c		-0.03 (-0.44, 0.39)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	13	12
Mean (SD)	69.23 (23.17)	76.57 (20.58)
Median	78.13	81.26
25th, 75th Percentile	59.38, 84.38	57.82, 95.32
Min, Max	25.0, 100.0	46.9, 100.0
Week 26		
n	13	11
Mean (SD)	77.82 (22.99)	79.55 (19.33)
Median	78.13	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	71.88, 96.43	71.88, 93.75
Min, Max	25.0, 100.0	34.4, 96.9
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	8.58 (10.96)	2.56 (30.29)
Median	6.25	0.00
25th, 75th Percentile	0.00, 18.30	-12.50, 31.25
Min, Max	-9.4, 31.3	-65.6, 43.8
Week 52		
n	13	11
Mean (SD)	72.84 (19.15)	74.15 (20.54)
Median	78.13	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 81.25	59.38, 93.75
Min, Max	28.1, 100.0	40.6, 100.0
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	3.60 (13.73)	-2.84 (11.13)
Median	3.13	0.00
25th, 75th Percentile	-3.12, 9.38	-15.63, 3.12
Min, Max	-25.0, 25.0	-18.8, 15.6
Difference in change from baseline (95%CI)		-6.45 (-17.17, 4.27)
P-value ^b		0.2254
Hedges'g (95% CI) ^c		-0.49 (-1.30, 0.33)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5368

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	10	15
Mean (SD)	59.65 (20.78)	65.36 (26.59)
Median	59.38	71.88
25th, 75th Percentile	37.50, 81.25	50.00, 90.63
Min, Max	34.4, 84.4	3.6, 93.8
Week 26		
n	10	11
Mean (SD)	58.44 (29.87)	69.61 (17.74)
Median	56.25	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 84.38	65.63, 81.25
Min, Max	18.8, 100.0	28.1, 90.6
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-1.21 (31.20)	4.91 (18.04)
Median	1.56	3.13
25th, 75th Percentile	-15.18, 18.75	-12.50, 13.84
Min, Max	-62.5, 37.5	-18.8, 46.4
Week 52		
n	9	12
Mean (SD)	63.84 (15.14)	58.60 (17.63)
Median	59.38	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 71.88	43.76, 73.44
Min, Max	46.4, 90.6	34.4, 93.8
Change from baseline to Week 52 ^a		
n	9	12
Mean (SD)	5.56 (15.76)	-3.05 (31.71)
Median	6.25	-8.71
25th, 75th Percentile	0.00, 15.63	-18.75, 7.81
Min, Max	-25.0, 25.0	-59.4, 65.6
Difference in change from baseline (95%CI)		-8.61 (-32.80, 15.58)
P-value ^b		0.4654
Hedges'g (95% CI) ^c		-0.32 (-1.18, 0.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	23	17
Mean (SD)	78.94 (19.61)	63.42 (22.12)
Median	84.38	56.25
25th, 75th Percentile	65.63, 96.88	50.00, 87.50
Min, Max	31.3, 100.0	21.9, 100.0
Week 26		
n	22	17
Mean (SD)	77.38 (17.92)	58.88 (21.65)
Median	81.25	57.14

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 90.63	46.88, 78.13
Min, Max	40.6, 100.0	18.8, 93.8
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	-1.68 (12.51)	-4.54 (24.23)
Median	0.00	-6.25
25th, 75th Percentile	-6.25, 5.80	-12.50, 6.25
Min, Max	-34.4, 18.8	-81.3, 31.3
Week 52		
n	23	17
Mean (SD)	75.41 (19.84)	61.35 (26.11)
Median	78.13	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 93.75	46.88, 81.25
Min, Max	34.4, 100.0	9.4, 100.0
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-2.98 (20.03)	-2.07 (28.93)
Median	-1.56	0.00
25th, 75th Percentile	-9.37, 9.37	-9.37, 12.50
Min, Max	-65.6, 28.1	-90.6, 40.6
Difference in change from baseline (95%CI)		0.91 (-14.98, 16.80)
P-value ^b		0.9084
Hedges'g (95% CI) ^c		0.04 (-0.60, 0.67)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	18	22
Mean (SD)	65.11 (20.82)	78.55 (22.77)
Median	68.75	87.50
25th, 75th Percentile	59.38, 78.13	56.25, 96.88
Min, Max	25.0, 96.9	15.6, 100.0
Week 26		
n	19	21
Mean (SD)	73.03 (21.20)	78.19 (23.63)
Median	75.00	87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 90.63	65.63, 93.75
Min, Max	25.0, 100.0	15.6, 100.0
Change from baseline to Week 26 ^a		
n	18	21
Mean (SD)	8.51 (14.09)	-0.09 (20.94)
Median	7.82	0.00
25th, 75th Percentile	0.00, 18.75	-3.13, 10.71
Min, Max	-18.8, 40.6	-65.6, 43.8
Week 52		
n	19	22
Mean (SD)	68.59 (19.53)	81.54 (20.18)
Median	71.88	87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 84.38	62.50, 96.88
Min, Max	28.1, 93.8	34.4, 100.0
Change from baseline to Week 52 ^a		
n	18	22
Mean (SD)	4.34 (14.27)	2.98 (13.06)
Median	4.69	0.00
25th, 75th Percentile	-3.13, 9.38	-3.13, 9.38
Min, Max	-21.9, 37.5	-21.9, 34.4
Difference in change from baseline (95%CI)		-1.36 (-10.12, 7.40)
P-value ^b		0.7552
Hedges'g (95% CI) ^c		-0.10 (-0.72, 0.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	8	5
Mean (SD)	73.83 (16.19)	77.50 (16.45)
Median	73.44	75.00
25th, 75th Percentile	65.63, 82.82	68.75, 90.63
Min, Max	46.9, 100.0	56.3, 96.9
Week 26		
n	8	5
Mean (SD)	77.74 (21.22)	82.50 (16.92)
Median	81.25	93.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 95.32	65.63, 93.75
Min, Max	43.8, 100.0	62.5, 96.9
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	3.90 (13.34)	5.00 (19.34)
Median	3.13	-3.12
25th, 75th Percentile	-3.13, 12.50	-3.13, 6.25
Min, Max	-18.8, 25.0	-12.5, 37.5
Week 52		
n	8	5
Mean (SD)	74.61 (17.63)	71.88 (21.76)
Median	73.44	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 89.07	62.50, 90.63
Min, Max	46.9, 100.0	40.6, 93.8
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	0.78 (15.82)	-5.62 (18.41)
Median	0.00	-6.25
25th, 75th Percentile	-7.81, 12.50	-6.25, 3.12
Min, Max	-25.0, 21.9	-34.4, 15.6
Difference in change from baseline (95%CI)		-6.40 (-27.49, 14.69)
P-value ^b		0.5177
Hedges'g (95% CI) ^c		-0.35 (-1.47, 0.78)
P-value for interaction term, treatment *[Baseline Height Z-score]		0.8381

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	19	19
Mean (SD)	65.30 (20.93)	76.32 (20.39)
Median	65.63	84.38
25th, 75th Percentile	50.00, 81.25	56.25, 93.75
Min, Max	25.0, 100.0	40.6, 100.0
Week 26		
n	19	17
Mean (SD)	68.73 (22.46)	66.73 (24.88)
Median	75.00	65.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 84.38	46.88, 87.50
Min, Max	18.8, 100.0	18.8, 100.0
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	3.43 (21.38)	-8.09 (21.51)
Median	3.12	-6.25
25th, 75th Percentile	-3.13, 18.30	-12.50, 0.00
Min, Max	-62.5, 37.5	-81.3, 18.8
Week 52		
n	18	18
Mean (SD)	73.09 (19.53)	66.67 (26.77)
Median	78.13	68.76

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 87.50	46.88, 93.75
Min, Max	28.1, 100.0	9.4, 100.0
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	7.47 (14.70)	-8.68 (28.25)
Median	9.38	0.00
25th, 75th Percentile	3.12, 15.63	-12.50, 9.37
Min, Max	-25.0, 28.1	-90.6, 25.0
Difference in change from baseline (95%CI)		-16.14 (-31.58, -0.70)
P-value ^b		0.0411
Hedges'g (95% CI) ^c		-0.70 (-1.37, -0.02)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	18	13
Mean (SD)	75.35 (21.49)	66.83 (29.82)
Median	79.69	75.00
25th, 75th Percentile	68.75, 87.50	56.25, 87.50
Min, Max	28.1, 100.0	15.6, 100.0
Week 26		
n	16	11
Mean (SD)	82.79 (21.36)	72.45 (25.91)
Median	89.07	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	75.00, 100.00	46.88, 93.75
Min, Max	28.1, 100.0	15.6, 93.8
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	6.22 (16.10)	2.84 (19.08)
Median	6.03	0.00
25th, 75th Percentile	0.00, 17.19	-9.37, 21.87
Min, Max	-34.4, 40.6	-31.3, 37.5
Week 52		
n	18	12
Mean (SD)	75.00 (18.25)	75.71 (23.16)
Median	76.57	84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 84.38	56.25, 95.32
Min, Max	34.4, 100.0	34.4, 100.0
Change from baseline to Week 52 ^a		
n	18	12
Mean (SD)	-0.35 (22.30)	9.56 (25.85)
Median	0.00	4.69
25th, 75th Percentile	-6.25, 9.38	-9.38, 23.44
Min, Max	-65.6, 37.5	-25.0, 65.6
Difference in change from baseline (95%CI)		9.91 (-8.23, 28.05)
P-value ^b		0.2727
Hedges'g (95% CI) ^c		0.41 (-0.34, 1.14)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	22	27
Mean (SD)	71.71 (19.85)	68.72 (22.83)
Median	75.00	68.75
25th, 75th Percentile	59.38, 84.38	50.00, 90.63
Min, Max	31.3, 100.0	3.6, 100.0
Week 26		
n	24	26
Mean (SD)	69.40 (21.49)	72.68 (20.20)
Median	75.00	73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 85.94	57.14, 93.75
Min, Max	31.3, 100.0	31.3, 100.0
Change from baseline to Week 26 ^a		
n	22	26
Mean (SD)	-1.26 (15.46)	4.09 (20.95)
Median	-1.57	3.12
25th, 75th Percentile	-6.25, 9.38	-3.12, 13.84
Min, Max	-40.6, 25.0	-65.6, 46.4
Week 52		
n	23	26
Mean (SD)	67.10 (18.82)	68.87 (21.77)
Median	68.75	67.19

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.2.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 84.38	56.25, 90.63
Min, Max	37.5, 96.9	25.0, 100.0
Change from baseline to Week 52 ^a		
n	21	26
Mean (SD)	-2.83 (12.54)	0.27 (16.20)
Median	-3.12	0.00
25th, 75th Percentile	-9.37, 3.12	-8.04, 9.37
Min, Max	-28.1, 21.9	-25.0, 37.1
Difference in change from baseline (95%CI)		3.10 (-5.58, 11.78)
P-value ^b		0.4756
Hedges'g (95% CI) ^c		0.21 (-0.37, 0.78)
P-value for interaction term, treatment * [Baseline AGV]		0.0233

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.1.2.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	40	44
Mean (SD)	70.85 (18.97)	70.14 (22.59)
Median	70.32	71.88
25th, 75th Percentile	59.38, 82.82	53.13, 90.63
Min, Max	25.0, 100.0	15.6, 100.0
Week 26		
n	40	41
Mean (SD)	74.37 (21.54)	72.33 (21.38)
Median	76.57	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.1.2.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	64.07, 90.63	59.38, 90.63
Min, Max	18.8, 100.0	15.6, 100.0
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	4.57 (19.43)	2.09 (13.42)
Median	6.25	0.00
25th, 75th Percentile	-3.13, 18.75	-6.25, 9.38
Min, Max	-62.5, 40.6	-18.8, 43.8
Week 52		
n	39	43
Mean (SD)	70.74 (19.90)	70.69 (22.24)
Median	71.88	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 87.50	50.00, 93.75
Min, Max	28.1, 100.0	31.3, 100.0
Change from baseline to Week 52 ^a		
n	38	43
Mean (SD)	0.08 (18.16)	1.10 (20.60)
Median	1.56	0.00
25th, 75th Percentile	-6.25, 9.38	-9.37, 9.38
Min, Max	-65.6, 28.1	-59.4, 65.6
Difference in change from baseline (95%CI)		1.02 (-7.62, 9.66)
P-value ^b		0.8152
Hedges'g (95% CI) ^c		0.05 (-0.38, 0.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	19	15
Mean (SD)	70.56 (24.66)	72.53 (27.52)
Median	78.13	75.00
25th, 75th Percentile	37.50, 87.50	50.00, 96.88
Min, Max	28.1, 100.0	3.6, 100.0
Week 26		
n	19	13
Mean (SD)	69.55 (23.97)	65.80 (26.63)
Median	75.00	65.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.1.2.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 90.63	50.00, 85.71
Min, Max	28.1, 100.0	18.8, 100.0
Change from baseline to Week 26 ^a		
n	18	13
Mean (SD)	-2.28 (12.72)	-6.59 (36.17)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 3.12	-15.63, 10.71
Min, Max	-34.4, 18.8	-81.3, 46.4
Week 52		
n	20	13
Mean (SD)	72.50 (17.10)	66.11 (28.23)
Median	78.13	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.2.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.94, 85.94	53.13, 87.50
Min, Max	40.6, 93.8	9.4, 100.0
Change from baseline to Week 52 ^a		
n	19	13
Mean (SD)	3.45 (14.95)	-6.28 (31.01)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 12.50	-18.75, 6.25
Min, Max	-25.0, 37.5	-90.6, 37.1
Difference in change from baseline (95%CI)		-9.74 (-29.38, 9.91)
P-value ^b		0.3087
Hedges'g (95% CI) ^c		-0.42 (-1.13, 0.30)
P-value for interaction term, treatment * [Ethnicity]		0.2166

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	32	30
Mean (SD)	72.82 (17.16)	73.44 (16.04)
Median	74.23	72.50
25th, 75th Percentile	60.00, 88.33	60.00, 88.33
Min, Max	31.7, 100.0	38.3, 100.0
Week 26		
n	31	27
Mean (SD)	75.11 (17.76)	70.86 (15.90)
Median	81.67	73.21

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 86.67	61.67, 81.67
Min, Max	31.7, 100.0	31.7, 96.7
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	1.50 (11.45)	-2.35 (19.45)
Median	0.00	0.00
25th, 75th Percentile	-6.66, 6.67	-10.00, 10.00
Min, Max	-21.7, 25.0	-65.0, 26.7
Week 52		
n	32	29
Mean (SD)	73.55 (17.23)	73.91 (15.43)
Median	75.84	78.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 88.33	68.33, 85.00
Min, Max	36.7, 95.0	33.3, 98.3
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	-0.26 (14.45)	0.58 (19.44)
Median	1.31	1.67
25th, 75th Percentile	-10.00, 8.34	-8.33, 13.34
Min, Max	-31.7, 26.7	-66.7, 28.3
Difference in change from baseline (95%CI)		0.84 (-7.98, 9.65)
P-value ^b		0.8502
Hedges'g (95% CI) ^c		0.05 (-0.46, 0.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	27	29
Mean (SD)	72.56 (16.91)	70.06 (12.35)
Median	73.33	68.33
25th, 75th Percentile	58.33, 88.33	63.33, 78.33
Min, Max	37.5, 100.0	43.3, 96.7
Week 26		
n	28	27
Mean (SD)	74.33 (14.54)	74.81 (14.45)
Median	76.67	78.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	63.34, 85.00	61.67, 85.00
Min, Max	46.7, 95.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	27	27
Mean (SD)	2.49 (9.95)	3.95 (15.31)
Median	3.33	6.67
25th, 75th Percentile	-5.00, 9.17	-5.00, 15.00
Min, Max	-11.7, 28.3	-30.0, 25.0
Week 52		
n	27	27
Mean (SD)	75.20 (17.64)	70.80 (11.99)
Median	81.67	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 90.00	60.00, 78.33
Min, Max	40.0, 100.0	51.7, 95.0
Change from baseline to Week 52 ^a		
n	26	27
Mean (SD)	2.55 (12.00)	-0.06 (11.83)
Median	3.34	0.00
25th, 75th Percentile	-3.33, 10.00	-5.00, 8.33
Min, Max	-28.3, 23.3	-30.0, 21.7
Difference in change from baseline (95%CI)		-2.61 (-9.18, 3.96)
P-value ^b		0.4290
Hedges'g (95% CI) ^c		-0.22 (-0.75, 0.33)
P-value for interaction term, treatment * [Sex]		0.5403

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	23	30
Mean (SD)	71.59 (17.68)	72.50 (14.63)
Median	73.33	72.50
25th, 75th Percentile	56.67, 88.33	63.33, 81.67
Min, Max	31.7, 96.7	38.3, 100.0
Week 26		
n	24	29
Mean (SD)	71.86 (18.11)	72.41 (17.37)
Median	80.84	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.17, 84.17	61.67, 85.00
Min, Max	31.7, 94.6	31.7, 100.0
Change from baseline to Week 26 ^a		
n	23	29
Mean (SD)	-0.23 (10.55)	0.34 (17.67)
Median	0.00	0.00
25th, 75th Percentile	-8.34, 5.00	-6.67, 11.67
Min, Max	-21.7, 21.7	-65.0, 25.0
Week 52		
n	22	30
Mean (SD)	74.62 (18.59)	71.94 (15.01)
Median	82.50	73.34

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 88.33	61.67, 80.00
Min, Max	36.7, 100.0	33.3, 98.3
Change from baseline to Week 52 ^a		
n	21	30
Mean (SD)	0.64 (12.71)	-0.56 (17.03)
Median	3.33	-0.83
25th, 75th Percentile	-3.33, 8.33	-6.66, 8.34
Min, Max	-26.7, 23.3	-66.7, 25.0
Difference in change from baseline (95%CI)		-1.19 (-10.00, 7.62)
P-value ^b		0.7868
Hedges'g (95% CI) ^c		-0.08 (-0.63, 0.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	24	17
Mean (SD)	73.51 (18.04)	67.06 (13.85)
Median	75.90	66.67
25th, 75th Percentile	60.00, 88.33	56.67, 75.00
Min, Max	37.5, 100.0	43.3, 88.3
Week 26		
n	23	15
Mean (SD)	75.58 (14.95)	71.11 (12.06)
Median	76.67	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Psychosocial summary score is the mean score (sum of items scored/number of items answered in the emotional, social and school functioning domains).

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 86.67	58.33, 81.67
Min, Max	46.7, 96.7	51.7, 91.7
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	1.70 (10.17)	3.00 (17.25)
Median	1.67	6.67
25th, 75th Percentile	-3.33, 9.17	-3.33, 15.00
Min, Max	-20.0, 25.0	-30.0, 25.0
Week 52		
n	24	15
Mean (SD)	72.67 (16.97)	71.56 (12.70)
Median	71.67	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.84, 89.17	60.00, 80.00
Min, Max	40.0, 98.3	43.3, 88.3
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	-0.84 (13.80)	3.44 (15.03)
Median	1.49	3.33
25th, 75th Percentile	-9.17, 7.50	-8.33, 15.00
Min, Max	-31.7, 26.7	-30.0, 28.3
Difference in change from baseline (95%CI)		4.29 (-5.24, 13.81)
P-value ^b		0.3676
Hedges'g (95% CI) ^c		0.29 (-0.36, 0.94)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	12	12
Mean (SD)	73.20 (14.02)	76.67 (13.28)
Median	70.00	75.00
25th, 75th Percentile	63.34, 85.00	64.17, 88.33
Min, Max	51.7, 93.3	61.7, 98.3
Week 26		
n	12	10
Mean (SD)	78.89 (14.47)	76.67 (13.05)
Median	80.84	76.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	70.00, 88.33
Min, Max	55.0, 100.0	56.7, 95.0
Change from baseline to Week 26 ^a		
n	11	10
Mean (SD)	7.12 (11.20)	-1.17 (19.53)
Median	6.67	4.17
25th, 75th Percentile	0.00, 16.66	-5.00, 8.34
Min, Max	-11.7, 28.3	-40.0, 26.7
Week 52		
n	13	11
Mean (SD)	76.80 (16.59)	74.85 (12.88)
Median	76.67	71.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.33, 86.67	61.67, 88.33
Min, Max	41.7, 96.7	60.0, 91.7
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	5.42 (13.65)	-1.82 (15.61)
Median	8.34	0.00
25th, 75th Percentile	-2.50, 15.84	-6.66, 3.34
Min, Max	-23.3, 23.3	-38.3, 23.3
Difference in change from baseline (95%CI)		-7.23 (-19.92, 5.45)
P-value ^b		0.2490
Hedges'g (95% CI) ^c		-0.48 (-1.30, 0.36)
P-value for interaction term, treatment * [Age at Baseline]		0.3436

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	46	47
Mean (SD)	74.24 (17.00)	71.45 (14.26)
Median	77.56	71.67
25th, 75th Percentile	60.00, 88.33	60.00, 81.67
Min, Max	31.7, 100.0	38.3, 100.0
Week 26		
n	46	43
Mean (SD)	74.99 (16.43)	72.05 (15.68)
Median	80.84	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 86.67	61.67, 85.00
Min, Max	31.7, 96.7	31.7, 100.0
Change from baseline to Week 26 ^a		
n	44	43
Mean (SD)	0.60 (10.93)	0.97 (17.43)
Median	0.00	1.66
25th, 75th Percentile	-8.33, 6.67	-6.67, 11.67
Min, Max	-21.7, 25.0	-65.0, 26.7
Week 52		
n	46	45
Mean (SD)	74.98 (17.87)	71.74 (14.49)
Median	77.50	71.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 90.00	61.67, 80.00
Min, Max	36.7, 100.0	33.3, 98.3
Change from baseline to Week 52 ^a		
n	44	45
Mean (SD)	-0.06 (13.62)	0.48 (17.43)
Median	1.49	1.67
25th, 75th Percentile	-9.17, 8.34	-6.66, 11.67
Min, Max	-31.7, 26.7	-66.7, 28.3
Difference in change from baseline (95%CI)		0.54 (-6.06, 7.14)
P-value ^b		0.8707
Hedges'g (95% CI) ^c		0.03 (-0.38, 0.45)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	13	12
Mean (SD)	67.24 (16.00)	73.05 (15.16)
Median	66.67	70.83
25th, 75th Percentile	56.67, 80.00	63.33, 88.33
Min, Max	37.5, 93.3	43.3, 96.7
Week 26		
n	13	11
Mean (SD)	73.85 (15.86)	75.91 (13.24)
Median	73.33	78.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 85.00	58.33, 88.33
Min, Max	46.7, 100.0	56.7, 91.7
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	6.60 (8.61)	0.15 (19.24)
Median	5.00	5.00
25th, 75th Percentile	0.00, 9.17	-5.00, 13.33
Min, Max	-3.3, 28.3	-40.0, 25.0
Week 52		
n	13	11
Mean (SD)	71.92 (15.47)	75.15 (10.94)
Median	75.00	76.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 85.00	65.00, 85.00
Min, Max	40.0, 95.0	60.0, 90.0
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	4.68 (12.18)	-0.60 (9.23)
Median	6.67	0.00
25th, 75th Percentile	1.67, 11.66	-6.66, 1.67
Min, Max	-28.3, 18.3	-11.7, 21.7
Difference in change from baseline (95%CI)		-5.28 (-14.58, 4.01)
P-value ^b		0.2508
Hedges'g (95% CI) ^c		-0.47 (-1.28, 0.35)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.3962

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	10	15
Mean (SD)	67.67 (16.85)	67.67 (12.11)
Median	59.17	65.00
25th, 75th Percentile	56.67, 88.33	60.00, 76.67
Min, Max	50.0, 93.3	43.3, 88.3
Week 26		
n	10	11
Mean (SD)	67.63 (16.92)	71.06 (12.52)
Median	66.67	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 75.00	58.33, 78.33
Min, Max	46.7, 94.6	53.3, 91.7
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-0.04 (11.31)	2.42 (20.53)
Median	0.00	10.00
25th, 75th Percentile	-8.34, 5.00	-20.00, 18.33
Min, Max	-20.0, 16.7	-30.0, 25.0
Week 52		
n	9	12
Mean (SD)	70.37 (21.75)	69.72 (11.78)
Median	75.00	72.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 85.00	60.00, 79.17
Min, Max	36.7, 95.0	51.7, 88.3
Change from baseline to Week 52 ^a		
n	9	12
Mean (SD)	0.74 (13.40)	1.39 (14.35)
Median	3.33	1.67
25th, 75th Percentile	-3.33, 5.00	-5.00, 10.00
Min, Max	-20.0, 23.3	-30.0, 23.3
Difference in change from baseline (95%CI)		0.65 (-12.23, 13.53)
P-value ^b		0.9175
Hedges'g (95% CI) ^c		0.04 (-0.82, 0.91)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	23	17
Mean (SD)	77.61 (14.54)	69.31 (14.59)
Median	80.00	68.33
25th, 75th Percentile	65.00, 88.33	58.33, 75.00
Min, Max	53.3, 100.0	45.0, 100.0
Week 26		
n	22	17
Mean (SD)	79.17 (12.42)	68.52 (15.70)
Median	82.50	73.21

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Psychosocial summary score is the mean score (sum of items scored/number of items answered in the emotional, social and school functioning domains).

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	73.33, 88.33	58.33, 80.00
Min, Max	45.0, 96.7	35.0, 95.0
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	0.16 (8.91)	-0.79 (21.39)
Median	0.00	-3.33
25th, 75th Percentile	-5.00, 5.00	-6.67, 15.00
Min, Max	-11.7, 18.3	-65.0, 26.7
Week 52		
n	23	17
Mean (SD)	77.10 (15.92)	66.37 (14.27)
Median	81.67	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	63.33, 88.33	60.00, 76.67
Min, Max	41.7, 100.0	33.3, 86.7
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-1.97 (13.86)	-2.94 (19.27)
Median	-0.83	-3.33
25th, 75th Percentile	-6.67, 6.67	-6.67, 3.34
Min, Max	-31.7, 23.3	-66.7, 28.3
Difference in change from baseline (95%CI)		-0.97 (-11.72, 9.77)
P-value ^b		0.8557
Hedges'g (95% CI) ^c		-0.06 (-0.69, 0.58)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	18	22
Mean (SD)	67.09 (18.29)	74.47 (15.26)
Median	65.00	75.84
25th, 75th Percentile	58.33, 80.00	63.33, 85.00
Min, Max	31.7, 100.0	38.3, 96.7
Week 26		
n	19	21
Mean (SD)	70.35 (17.75)	75.79 (16.33)
Median	76.67	81.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 83.33	66.67, 86.67
Min, Max	31.7, 95.0	31.7, 100.0
Change from baseline to Week 26 ^a		
n	18	21
Mean (SD)	4.12 (13.23)	1.83 (12.60)
Median	3.34	5.00
25th, 75th Percentile	-3.33, 10.00	-5.00, 8.34
Min, Max	-21.7, 28.3	-40.0, 25.0
Week 52		
n	19	22
Mean (SD)	71.62 (17.80)	78.03 (13.06)
Median	71.67	79.17

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 86.67	70.00, 85.00
Min, Max	40.0, 98.3	51.7, 98.3
Change from baseline to Week 52 ^a		
n	18	22
Mean (SD)	5.45 (11.91)	3.56 (12.94)
Median	7.50	4.17
25th, 75th Percentile	0.00, 11.67	-3.34, 13.34
Min, Max	-18.3, 26.7	-28.3, 25.0
Difference in change from baseline (95%CI)		-1.89 (-9.93, 6.15)
P-value ^b		0.6367
Hedges'g (95% CI) ^c		-0.15 (-0.77, 0.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	8	5
Mean (SD)	77.50 (17.39)	80.67 (12.61)
Median	83.33	76.67
25th, 75th Percentile	65.83, 91.67	73.33, 88.33
Min, Max	45.0, 93.3	66.7, 98.3
Week 26		
n	8	5
Mean (SD)	81.88 (16.84)	79.00 (12.73)
Median	85.84	78.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	76.67, 91.67	70.00, 91.67
Min, Max	46.7, 100.0	63.3, 91.7
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	4.38 (7.86)	-1.67 (20.17)
Median	3.34	3.34
25th, 75th Percentile	-0.83, 10.01	-13.34, 5.00
Min, Max	-6.7, 16.7	-28.3, 25.0
Week 52		
n	8	5
Mean (SD)	77.08 (15.78)	74.67 (13.61)
Median	80.00	68.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	64.17, 90.00	66.67, 88.33
Min, Max	53.3, 95.0	60.0, 90.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-0.42 (14.77)	-6.00 (21.65)
Median	0.00	-6.66
25th, 75th Percentile	-6.67, 8.34	-8.34, 1.67
Min, Max	-28.3, 21.7	-38.3, 21.7
Difference in change from baseline (95%CI)		-5.59 (-27.65, 16.48)
P-value ^b		0.5886
Hedges'g (95% CI) ^c		-0.30 (-1.41, 0.84)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.9479

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	19	19
Mean (SD)	70.40 (17.71)	75.88 (12.75)
Median	63.33	75.00
25th, 75th Percentile	56.67, 88.33	68.33, 85.00
Min, Max	37.5, 100.0	56.7, 100.0
Week 26		
n	19	17
Mean (SD)	71.58 (16.49)	72.65 (17.52)
Median	73.33	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.67, 86.67	61.67, 81.67
Min, Max	45.0, 96.7	35.0, 100.0
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	1.18 (10.12)	-2.65 (22.26)
Median	3.34	-1.66
25th, 75th Percentile	-8.33, 9.17	-13.33, 8.34
Min, Max	-20.0, 16.7	-65.0, 26.7
Week 52		
n	18	18
Mean (SD)	72.54 (19.86)	72.69 (17.00)
Median	77.50	73.34

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 88.33	60.00, 86.67
Min, Max	36.7, 98.3	33.3, 98.3
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	1.38 (13.62)	-3.15 (20.15)
Median	4.59	0.84
25th, 75th Percentile	-3.33, 10.00	-5.00, 5.00
Min, Max	-31.7, 23.3	-66.7, 20.0
Difference in change from baseline (95%CI)		-4.52 (-16.17, 7.12)
P-value ^b		0.4354
Hedges'g (95% CI) ^c		-0.26 (-0.91, 0.40)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	18	13
Mean (SD)	71.02 (16.91)	68.59 (15.27)
Median	70.00	66.67
25th, 75th Percentile	63.33, 88.33	56.67, 78.33
Min, Max	31.7, 93.3	38.3, 88.3
Week 26		
n	16	11
Mean (SD)	77.40 (17.36)	71.52 (18.65)
Median	82.50	78.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.84, 90.00	55.00, 85.00
Min, Max	31.7, 100.0	31.7, 91.7
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	5.63 (11.17)	3.18 (17.02)
Median	2.50	6.67
25th, 75th Percentile	-2.50, 12.51	-6.66, 18.34
Min, Max	-11.7, 28.3	-30.0, 25.0
Week 52		
n	18	12
Mean (SD)	74.26 (17.82)	72.64 (15.07)
Median	76.67	78.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	61.67, 83.33
Min, Max	40.0, 96.7	43.3, 88.3
Change from baseline to Week 52 ^a		
n	18	12
Mean (SD)	3.24 (15.20)	4.58 (16.88)
Median	2.51	6.67
25th, 75th Percentile	-5.00, 15.00	-7.50, 17.50
Min, Max	-28.3, 26.7	-30.0, 28.3
Difference in change from baseline (95%CI)		1.34 (-10.78, 13.47)
P-value ^b		0.8225
Hedges'g (95% CI) ^c		0.08 (-0.65, 0.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	22	27
Mean (SD)	76.06 (16.42)	70.43 (14.78)
Median	79.17	68.33
25th, 75th Percentile	60.00, 88.33	60.00, 80.00
Min, Max	45.0, 100.0	43.3, 96.7
Week 26		
n	24	26
Mean (SD)	75.47 (15.39)	73.52 (12.31)
Median	81.67	74.17

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Psychosocial summary score is the mean score (sum of items scored/number of items answered in the emotional, social and school functioning domains).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.34, 85.84	65.00, 83.33
Min, Max	41.7, 94.6	45.0, 91.7
Change from baseline to Week 26 ^a		
n	22	26
Mean (SD)	-0.01 (10.60)	2.05 (14.60)
Median	-0.83	5.83
25th, 75th Percentile	-8.33, 4.64	-5.00, 11.67
Min, Max	-21.7, 21.7	-40.0, 25.0
Week 52		
n	23	26
Mean (SD)	75.73 (15.26)	72.12 (11.17)
Median	76.67	70.84

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 90.00	65.00, 80.00
Min, Max	51.7, 100.0	48.3, 93.3
Change from baseline to Week 52 ^a		
n	21	26
Mean (SD)	-1.19 (11.62)	0.64 (12.26)
Median	3.33	0.84
25th, 75th Percentile	-10.00, 5.00	-6.67, 8.33
Min, Max	-26.7, 18.3	-28.3, 25.0
Difference in change from baseline (95%CI)		1.83 (-5.25, 8.91)
P-value ^b		0.6050
Hedges'g (95% CI) ^c		0.15 (-0.43, 0.73)
P-value for interaction term, treatment * [Baseline AGV]		0.5913

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	40	44
Mean (SD)	71.94 (16.22)	71.55 (12.73)
Median	71.67	71.67
25th, 75th Percentile	59.17, 88.33	62.50, 80.00
Min, Max	37.5, 100.0	38.3, 98.3
Week 26		
n	40	41
Mean (SD)	74.66 (15.39)	73.13 (14.02)
Median	78.34	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 85.84	63.33, 83.33
Min, Max	41.7, 96.7	31.7, 100.0
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	3.04 (11.01)	1.87 (14.00)
Median	3.34	1.66
25th, 75th Percentile	-5.00, 11.67	-5.00, 11.67
Min, Max	-21.7, 25.0	-30.0, 26.7
Week 52		
n	39	43
Mean (SD)	73.40 (17.56)	73.02 (12.32)
Median	75.00	71.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 88.33	65.00, 81.67
Min, Max	36.7, 100.0	43.3, 98.3
Change from baseline to Week 52 ^a		
n	38	43
Mean (SD)	0.97 (14.28)	1.59 (13.11)
Median	3.33	1.67
25th, 75th Percentile	-10.00, 10.00	-5.00, 8.34
Min, Max	-31.7, 26.7	-38.3, 28.3
Difference in change from baseline (95%CI)		0.62 (-5.44, 6.68)
P-value ^b		0.8392
Hedges'g (95% CI) ^c		0.04 (-0.39, 0.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	19	15
Mean (SD)	74.30 (18.62)	72.44 (18.76)
Median	80.00	71.67
25th, 75th Percentile	56.67, 90.00	56.67, 88.33
Min, Max	31.7, 100.0	43.3, 100.0
Week 26		
n	19	13
Mean (SD)	74.91 (18.17)	71.91 (19.01)
Median	81.67	73.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.3.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	61.67, 88.33	58.33, 90.00
Min, Max	31.7, 100.0	35.0, 96.7
Change from baseline to Week 26 ^a		
n	18	13
Mean (SD)	-0.37 (9.81)	-2.57 (26.50)
Median	-0.83	8.34
25th, 75th Percentile	-6.66, 3.33	-6.67, 11.67
Min, Max	-20.0, 28.3	-65.0, 25.0
Week 52		
n	20	13
Mean (SD)	76.07 (17.04)	70.38 (18.45)
Median	84.17	76.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.3.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.84, 89.17	60.00, 85.00
Min, Max	40.0, 95.0	33.3, 93.3
Change from baseline to Week 52 ^a		
n	19	13
Mean (SD)	1.12 (11.63)	-4.10 (23.61)
Median	1.67	-3.33
25th, 75th Percentile	-3.33, 6.67	-11.66, 11.66
Min, Max	-28.3, 23.3	-66.7, 21.7
Difference in change from baseline (95%CI)		-5.23 (-20.21, 9.76)
P-value ^b		0.4705
Hedges'g (95% CI) ^c		-0.29 (-1.00, 0.42)
P-value for interaction term, treatment * [Ethnicity]		0.3529

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	32	30
Mean (SD)	77.34 (19.05)	77.83 (17.75)
Median	77.50	85.00
25th, 75th Percentile	65.00, 95.00	60.00, 90.00
Min, Max	40.0, 100.0	40.0, 100.0
Week 26		
n	31	27
Mean (SD)	78.55 (17.66)	76.67 (21.57)
Median	85.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.1.4.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	60.00, 95.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	0.33 (12.66)	-1.30 (19.44)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-20.0, 40.0	-70.0, 40.0
Week 52		
n	32	29
Mean (SD)	79.22 (22.15)	81.55 (14.58)
Median	90.00	85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 100.00	75.00, 90.00
Min, Max	30.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	0.81 (19.54)	3.45 (21.30)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 15.00
Min, Max	-55.0, 40.0	-65.0, 40.0
Difference in change from baseline (95%CI)		2.64 (-7.91, 13.20)
P-value ^b		0.6182
Hedges'g (95% CI) ^c		0.13 (-0.38, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	27	29
Mean (SD)	73.33 (18.81)	70.86 (12.33)
Median	75.00	70.00
25th, 75th Percentile	55.00, 90.00	65.00, 75.00
Min, Max	35.0, 100.0	45.0, 100.0
Week 26		
n	28	27
Mean (SD)	74.73 (17.38)	76.67 (16.81)
Median	77.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	57.50, 88.75	65.00, 95.00
Min, Max	40.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	27	27
Mean (SD)	2.13 (13.56)	5.56 (16.01)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	0.00, 20.00
Min, Max	-20.0, 35.0	-35.0, 35.0
Week 52		
n	27	27
Mean (SD)	77.96 (17.56)	75.00 (14.28)
Median	85.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 95.00	65.00, 90.00
Min, Max	45.0, 100.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	26	27
Mean (SD)	5.00 (12.33)	3.89 (12.58)
Median	5.00	5.00
25th, 75th Percentile	0.00, 10.00	-5.00, 15.00
Min, Max	-25.0, 30.0	-25.0, 30.0
Difference in change from baseline (95%CI)		-1.11 (-7.98, 5.76)
P-value ^b		0.7468
Hedges'g (95% CI) ^c		-0.09 (-0.63, 0.45)
P-value for interaction term, treatment * [Sex]		0.5631

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	23	30
Mean (SD)	76.96 (17.24)	72.83 (17.25)
Median	75.00	70.00
25th, 75th Percentile	65.00, 90.00	60.00, 90.00
Min, Max	45.0, 100.0	40.0, 100.0
Week 26		
n	24	29
Mean (SD)	76.56 (17.47)	73.97 (21.73)
Median	80.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 90.00	60.00, 95.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	23	29
Mean (SD)	-0.98 (13.12)	0.86 (19.37)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 5.00	-10.00, 10.00
Min, Max	-20.0, 30.0	-70.0, 35.0
Week 52		
n	22	30
Mean (SD)	80.45 (20.52)	77.33 (16.12)
Median	85.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 100.00	70.00, 90.00
Min, Max	30.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	21	30
Mean (SD)	0.95 (13.10)	4.50 (20.48)
Median	0.00	5.00
25th, 75th Percentile	0.00, 10.00	-5.00, 20.00
Min, Max	-35.0, 20.0	-65.0, 35.0
Difference in change from baseline (95%CI)		3.55 (-5.91, 13.01)
P-value ^b		0.4547
Hedges'g (95% CI) ^c		0.20 (-0.36, 0.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	24	17
Mean (SD)	73.96 (22.07)	72.35 (12.88)
Median	77.50	70.00
25th, 75th Percentile	57.50, 95.00	65.00, 80.00
Min, Max	35.0, 100.0	55.0, 100.0
Week 26		
n	23	15
Mean (SD)	73.26 (18.13)	78.00 (16.78)
Median	75.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	65.00, 95.00
Min, Max	40.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	-0.87 (10.62)	5.00 (20.18)
Median	-5.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 25.00
Min, Max	-20.0, 20.0	-35.0, 40.0
Week 52		
n	24	15
Mean (SD)	75.21 (21.03)	78.00 (12.36)
Median	75.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 95.00	65.00, 90.00
Min, Max	40.0, 100.0	60.0, 95.0
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	1.25 (19.24)	5.00 (15.47)
Median	5.00	5.00
25th, 75th Percentile	-7.50, 12.50	-5.00, 15.00
Min, Max	-55.0, 40.0	-20.0, 40.0
Difference in change from baseline (95%CI)		3.75 (-8.19, 15.69)
P-value ^b		0.5285
Hedges'g (95% CI) ^c		0.21 (-0.44, 0.85)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	12	12
Mean (SD)	75.83 (16.07)	81.25 (13.84)
Median	75.00	85.00
25th, 75th Percentile	62.50, 90.00	70.00, 92.50
Min, Max	55.0, 100.0	60.0, 100.0
Week 26		
n	12	10
Mean (SD)	83.75 (15.39)	82.50 (13.79)
Median	87.50	82.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	72.50, 95.00	70.00, 95.00
Min, Max	55.0, 100.0	60.0, 100.0
Change from baseline to Week 26 ^a		
n	11	10
Mean (SD)	10.00 (14.66)	1.50 (9.14)
Median	5.00	0.00
25th, 75th Percentile	0.00, 15.00	0.00, 10.00
Min, Max	-5.0, 40.0	-15.0, 15.0
Week 52		
n	13	11
Mean (SD)	81.92 (17.62)	81.82 (14.19)
Median	85.00	85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	75.00, 95.00	65.00, 95.00
Min, Max	45.0, 100.0	65.0, 100.0
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	8.75 (16.53)	-0.45 (10.36)
Median	5.00	0.00
25th, 75th Percentile	0.00, 20.00	-5.00, 5.00
Min, Max	-25.0, 35.0	-25.0, 15.0
Difference in change from baseline (95%CI)		-9.20 (-21.30, 2.89)
P-value ^b		0.1286
Hedges'g (95% CI) ^c		-0.64 (-1.47, 0.21)
P-value for interaction term, treatment * [Age at Baseline]		0.2856

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	46	47
Mean (SD)	77.39 (18.94)	74.04 (16.34)
Median	82.50	70.00
25th, 75th Percentile	65.00, 95.00	60.00, 90.00
Min, Max	40.0, 100.0	40.0, 100.0
Week 26		
n	46	43
Mean (SD)	77.55 (17.59)	75.47 (20.29)
Median	82.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	60.00, 95.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	44	43
Mean (SD)	0.06 (13.23)	1.51 (19.29)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 7.50	-10.00, 15.00
Min, Max	-20.0, 40.0	-70.0, 40.0
Week 52		
n	46	45
Mean (SD)	79.24 (20.74)	78.44 (15.03)
Median	87.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.4.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	70.00, 90.00
Min, Max	30.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	44	45
Mean (SD)	1.25 (16.95)	4.22 (19.13)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 15.00
Min, Max	-55.0, 40.0	-65.0, 40.0
Difference in change from baseline (95%CI)		2.97 (-4.65, 10.59)
P-value ^b		0.4403
Hedges'g (95% CI) ^c		0.16 (-0.25, 0.58)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	13	12
Mean (SD)	68.85 (17.81)	75.83 (12.76)
Median	65.00	70.00
25th, 75th Percentile	60.00, 80.00	65.00, 87.50
Min, Max	35.0, 100.0	60.0, 100.0
Week 26		
n	13	11
Mean (SD)	73.85 (17.46)	81.36 (13.62)
Median	75.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	70.00, 95.00
Min, Max	50.0, 100.0	60.0, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	5.00 (11.90)	4.55 (11.93)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 10.00	0.00, 10.00
Min, Max	-10.0, 35.0	-10.0, 30.0
Week 52		
n	13	11
Mean (SD)	76.54 (17.84)	78.18 (13.83)
Median	85.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 85.00	65.00, 90.00
Min, Max	45.0, 100.0	60.0, 95.0
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	7.69 (15.09)	1.36 (8.09)
Median	10.00	0.00
25th, 75th Percentile	0.00, 15.00	-5.00, 5.00
Min, Max	-25.0, 30.0	-10.0, 20.0
Difference in change from baseline (95%CI)		-6.33 (-16.87, 4.21)
P-value ^b		0.2261
Hedges'g (95% CI) ^c		-0.49 (-1.30, 0.33)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.2407

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	10	15
Mean (SD)	72.50 (19.76)	73.33 (13.18)
Median	70.00	75.00
25th, 75th Percentile	55.00, 90.00	65.00, 80.00
Min, Max	40.0, 100.0	45.0, 100.0
Week 26		
n	10	11
Mean (SD)	74.75 (14.65)	74.55 (18.09)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	65.00, 90.00
Min, Max	55.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	2.25 (16.60)	1.82 (18.61)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 15.00	-10.00, 25.00
Min, Max	-20.0, 30.0	-35.0, 25.0
Week 52		
n	9	12
Mean (SD)	74.44 (23.38)	77.50 (14.85)
Median	80.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	67.50, 85.00
Min, Max	30.0, 100.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	9	12
Mean (SD)	0.00 (13.69)	2.92 (7.22)
Median	5.00	5.00
25th, 75th Percentile	0.00, 5.00	-2.50, 7.50
Min, Max	-35.0, 10.0	-10.0, 15.0
Difference in change from baseline (95%CI)		2.92 (-6.72, 12.56)
P-value ^b		0.5341
Hedges'g (95% CI) ^c		0.27 (-0.60, 1.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	23	17
Mean (SD)	80.43 (16.51)	75.00 (16.20)
Median	85.00	70.00
25th, 75th Percentile	65.00, 95.00	65.00, 90.00
Min, Max	45.0, 100.0	50.0, 100.0
Week 26		
n	22	17
Mean (SD)	81.59 (16.21)	75.29 (17.27)
Median	87.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 95.00	70.00, 85.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	-0.24 (12.99)	0.29 (24.40)
Median	-5.00	0.00
25th, 75th Percentile	-5.00, 5.00	-10.00, 10.00
Min, Max	-20.0, 40.0	-70.0, 40.0
Week 52		
n	23	17
Mean (SD)	82.17 (20.22)	76.18 (15.67)
Median	90.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 100.00	65.00, 90.00
Min, Max	40.0, 100.0	35.0, 95.0
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	0.23 (18.16)	1.18 (23.55)
Median	0.00	-5.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 20.00
Min, Max	-55.0, 35.0	-65.0, 40.0
Difference in change from baseline (95%CI)		0.95 (-12.57, 14.47)
P-value ^b		0.8877
Hedges'g (95% CI) ^c		0.04 (-0.59, 0.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	18	22
Mean (SD)	67.22 (17.84)	74.09 (17.50)
Median	65.00	70.00
25th, 75th Percentile	55.00, 80.00	60.00, 90.00
Min, Max	35.0, 100.0	40.0, 100.0
Week 26		
n	19	21
Mean (SD)	69.21 (17.50)	78.10 (22.44)
Median	75.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	60.00, 100.00
Min, Max	40.0, 95.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	18	21
Mean (SD)	2.78 (13.64)	3.57 (12.06)
Median	2.50	0.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 10.00
Min, Max	-20.0, 35.0	-15.0, 30.0
Week 52		
n	19	22
Mean (SD)	76.32 (18.09)	80.91 (14.85)
Median	85.00	85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	70.00, 95.00
Min, Max	45.0, 100.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	18	22
Mean (SD)	10.56 (15.52)	6.82 (16.73)
Median	12.50	7.50
25th, 75th Percentile	0.00, 20.00	0.00, 20.00
Min, Max	-25.0, 40.0	-30.0, 35.0
Difference in change from baseline (95%CI)		-3.74 (-14.16, 6.69)
P-value ^b		0.4723
Hedges'g (95% CI) ^c		-0.23 (-0.85, 0.40)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	8	5
Mean (SD)	83.75 (21.84)	77.00 (15.65)
Median	92.50	70.00
25th, 75th Percentile	72.50, 100.00	70.00, 85.00
Min, Max	40.0, 100.0	60.0, 100.0
Week 26		
n	8	5
Mean (SD)	83.75 (19.78)	80.00 (16.96)
Median	92.50	85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	67.50, 100.00	65.00, 90.00
Min, Max	50.0, 100.0	60.0, 100.0
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	0.00 (7.07)	3.00 (16.81)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 5.00	-5.00, 5.00
Min, Max	-10.0, 10.0	-15.0, 30.0
Week 52		
n	8	5
Mean (SD)	78.75 (22.16)	77.00 (12.55)
Median	82.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	57.50, 100.00	65.00, 90.00
Min, Max	50.0, 100.0	65.0, 90.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-5.00 (13.09)	0.00 (16.58)
Median	0.00	5.00
25th, 75th Percentile	-15.00, 2.50	-5.00, 5.00
Min, Max	-25.0, 10.0	-25.0, 20.0
Difference in change from baseline (95%CI)		5.00 (-13.14, 23.14)
P-value ^b		0.5565
Hedges'g (95% CI) ^c		0.32 (-0.81, 1.44)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.8178

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	19	19
Mean (SD)	72.89 (21.04)	79.21 (16.85)
Median	70.00	85.00
25th, 75th Percentile	60.00, 95.00	65.00, 95.00
Min, Max	35.0, 100.0	45.0, 100.0
Week 26		
n	19	17
Mean (SD)	73.16 (17.01)	77.06 (21.00)
Median	75.00	85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	65.00, 90.00
Min, Max	50.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	0.26 (12.64)	-3.53 (22.55)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-15.00, 10.00
Min, Max	-20.0, 20.0	-70.0, 30.0
Week 52		
n	18	18
Mean (SD)	75.00 (24.37)	77.78 (18.41)
Median	80.00	82.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 95.00	65.00, 90.00
Min, Max	30.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	1.67 (19.17)	-1.94 (20.73)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 10.00
Min, Max	-55.0, 25.0	-65.0, 30.0
Difference in change from baseline (95%CI)		-3.61 (-17.14, 9.92)
P-value ^b		0.5910
Hedges'g (95% CI) ^c		-0.18 (-0.83, 0.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	18	13
Mean (SD)	74.17 (17.26)	71.15 (17.70)
Median	70.00	70.00
25th, 75th Percentile	60.00, 90.00	60.00, 80.00
Min, Max	40.0, 100.0	40.0, 100.0
Week 26		
n	16	11
Mean (SD)	79.38 (19.31)	76.36 (25.11)
Median	87.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	67.50, 95.00	60.00, 100.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	4.69 (15.43)	7.27 (22.51)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-10.00, 30.00
Min, Max	-20.0, 40.0	-35.0, 40.0
Week 52		
n	18	12
Mean (SD)	80.28 (18.27)	81.67 (16.42)
Median	85.00	90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	67.50, 95.00
Min, Max	45.0, 100.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	18	12
Mean (SD)	6.11 (18.19)	10.42 (15.88)
Median	0.00	7.50
25th, 75th Percentile	0.00, 15.00	-2.50, 20.00
Min, Max	-25.0, 40.0	-10.0, 40.0
Difference in change from baseline (95%CI)		4.31 (-8.92, 17.53)
P-value ^b		0.5102
Hedges'g (95% CI) ^c		0.24 (-0.49, 0.97)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	22	27
Mean (SD)	78.86 (18.58)	72.59 (13.26)
Median	85.00	70.00
25th, 75th Percentile	65.00, 95.00	65.00, 80.00
Min, Max	40.0, 100.0	50.0, 100.0
Week 26		
n	24	26
Mean (SD)	77.81 (16.86)	76.54 (15.61)
Median	82.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	70.00, 90.00
Min, Max	45.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	22	26
Mean (SD)	-0.57 (11.44)	3.65 (11.01)
Median	-1.25	0.00
25th, 75th Percentile	-5.00, 5.00	0.00, 10.00
Min, Max	-20.0, 30.0	-15.0, 25.0
Week 52		
n	23	26
Mean (SD)	80.22 (17.99)	77.31 (10.88)
Median	85.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 100.00	70.00, 85.00
Min, Max	50.0, 100.0	55.0, 95.0
Change from baseline to Week 52 ^a		
n	21	26
Mean (SD)	0.71 (12.87)	4.42 (14.99)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 15.00
Min, Max	-25.0, 20.0	-30.0, 35.0
Difference in change from baseline (95%CI)		3.71 (-4.62, 12.03)
P-value ^b		0.3743
Hedges'g (95% CI) ^c		0.26 (-0.32, 0.83)
P-value for interaction term, treatment * [Baseline AGV]		0.5487

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	40	44
Mean (SD)	72.88 (18.74)	74.20 (16.24)
Median	72.50	70.00
25th, 75th Percentile	62.50, 90.00	65.00, 87.50
Min, Max	35.0, 100.0	40.0, 100.0
Week 26		
n	40	41
Mean (SD)	75.94 (17.09)	77.20 (18.84)
Median	82.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	65.00, 95.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	3.53 (12.80)	3.17 (15.56)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 15.00	-10.00, 10.00
Min, Max	-20.0, 40.0	-35.0, 40.0
Week 52		
n	39	43
Mean (SD)	76.67 (20.91)	78.84 (14.09)
Median	85.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	65.00, 90.00
Min, Max	30.0, 100.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	38	43
Mean (SD)	3.82 (18.14)	4.53 (15.31)
Median	5.00	5.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 15.00
Min, Max	-55.0, 40.0	-30.0, 40.0
Difference in change from baseline (95%CI)		0.72 (-6.68, 8.12)
P-value ^b		0.8471
Hedges'g (95% CI) ^c		0.04 (-0.39, 0.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.4.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	19	15
Mean (SD)	81.05 (18.45)	75.00 (14.02)
Median	80.00	75.00
25th, 75th Percentile	60.00, 100.00	65.00, 90.00
Min, Max	55.0, 100.0	50.0, 100.0
Week 26		
n	19	13
Mean (SD)	78.42 (18.64)	75.00 (20.82)
Median	80.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 95.00	60.00, 95.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	18	13
Mean (SD)	-3.89 (12.31)	-1.15 (24.59)
Median	-2.50	0.00
25th, 75th Percentile	-10.00, 0.00	-10.00, 10.00
Min, Max	-20.0, 35.0	-70.0, 30.0
Week 52		
n	20	13
Mean (SD)	82.50 (18.03)	76.92 (17.02)
Median	87.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.4.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 100.00	75.00, 85.00
Min, Max	50.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	19	13
Mean (SD)	0.53 (13.32)	0.77 (23.88)
Median	0.00	10.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 10.00
Min, Max	-25.0, 30.0	-65.0, 25.0
Difference in change from baseline (95%CI)		0.24 (-15.14, 15.62)
P-value ^b		0.9738
Hedges'g (95% CI) ^c		0.01 (-0.69, 0.72)
P-value for interaction term, treatment * [Ethnicity]		0.9478

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	32	30
Mean (SD)	67.23 (20.73)	69.50 (22.41)
Median	70.00	65.00
25th, 75th Percentile	53.13, 87.50	55.00, 90.00
Min, Max	10.0, 100.0	25.0, 100.0
Week 26		
n	31	27
Mean (SD)	70.32 (19.70)	65.51 (20.48)
Median	75.00	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.5.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 80.00	50.00, 80.00
Min, Max	10.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	2.63 (16.62)	-2.82 (24.48)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-15.00, 10.00
Min, Max	-40.0, 40.0	-75.0, 45.0
Week 52		
n	32	29
Mean (SD)	69.53 (18.85)	70.34 (21.71)
Median	70.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.5.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 82.50	55.00, 85.00
Min, Max	35.0, 100.0	20.0, 100.0
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	1.09 (16.77)	1.55 (24.79)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 20.00
Min, Max	-35.0, 30.0	-80.0, 40.0
Difference in change from baseline (95%CI)		0.46 (-10.59, 11.52)
P-value ^b		0.9333
Hedges'g (95% CI) ^c		0.02 (-0.48, 0.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.5.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	27	29
Mean (SD)	66.20 (21.57)	64.83 (16.56)
Median	65.00	60.00
25th, 75th Percentile	50.00, 85.00	55.00, 75.00
Min, Max	12.5, 100.0	35.0, 100.0
Week 26		
n	28	27
Mean (SD)	72.32 (18.68)	72.41 (18.21)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	55.00, 85.00
Min, Max	35.0, 100.0	35.0, 100.0
Change from baseline to Week 26 ^a		
n	27	27
Mean (SD)	6.57 (14.30)	6.67 (18.66)
Median	5.00	10.00
25th, 75th Percentile	0.00, 15.00	0.00, 20.00
Min, Max	-15.0, 40.0	-40.0, 35.0
Week 52		
n	27	27
Mean (SD)	70.56 (23.05)	63.33 (17.32)
Median	75.00	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.005.001_qs_sum_ovr_ped_care_soc_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	50.00, 70.00
Min, Max	25.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	26	27
Mean (SD)	3.94 (17.32)	-2.41 (14.50)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 15.00	-10.00, 5.00
Min, Max	-30.0, 45.0	-50.0, 25.0
Difference in change from baseline (95%CI)		-6.35 (-15.15, 2.45)
P-value ^b		0.1534
Hedges'g (95% CI) ^c		-0.39 (-0.93, 0.15)
P-value for interaction term, treatment * [Sex]		0.3393

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	23	30
Mean (SD)	65.00 (22.91)	68.50 (19.08)
Median	60.00	62.50
25th, 75th Percentile	45.00, 90.00	55.00, 80.00
Min, Max	10.0, 100.0	25.0, 100.0
Week 26		
n	24	29
Mean (SD)	66.46 (22.19)	67.89 (21.11)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 80.00	55.00, 85.00
Min, Max	10.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	23	29
Mean (SD)	1.09 (16.99)	0.13 (21.34)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-40.0, 35.0	-75.0, 35.0
Week 52		
n	22	30
Mean (SD)	72.05 (20.33)	66.00 (20.53)
Median	77.50	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	50.00, 80.00
Min, Max	40.0, 100.0	20.0, 100.0
Change from baseline to Week 52 ^a		
n	21	30
Mean (SD)	4.52 (18.36)	-2.50 (20.88)
Median	0.00	-5.00
25th, 75th Percentile	-5.00, 10.00	-10.00, 10.00
Min, Max	-35.0, 45.0	-80.0, 40.0
Difference in change from baseline (95%CI)		-7.02 (-18.40, 4.35)
P-value ^b		0.2204
Hedges'g (95% CI) ^c		-0.35 (-0.91, 0.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	24	17
Mean (SD)	65.99 (22.54)	61.47 (18.44)
Median	70.00	60.00
25th, 75th Percentile	50.00, 85.00	50.00, 70.00
Min, Max	12.5, 100.0	25.0, 100.0
Week 26		
n	23	15
Mean (SD)	73.26 (16.56)	67.00 (17.81)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	50.00, 85.00
Min, Max	35.0, 100.0	35.0, 90.0
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	7.23 (15.14)	4.33 (20.34)
Median	5.00	10.00
25th, 75th Percentile	0.00, 15.00	-15.00, 20.00
Min, Max	-20.0, 40.0	-40.0, 30.0
Week 52		
n	24	15
Mean (SD)	64.38 (20.76)	62.33 (18.50)
Median	65.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 77.50	45.00, 80.00
Min, Max	25.0, 100.0	30.0, 90.0
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	-1.61 (17.19)	-0.33 (19.13)
Median	-2.50	0.00
25th, 75th Percentile	-15.00, 13.75	-10.00, 15.00
Min, Max	-30.0, 30.0	-50.0, 20.0
Difference in change from baseline (95%CI)		1.28 (-10.69, 13.25)
P-value ^b		0.8295
Hedges'g (95% CI) ^c		0.07 (-0.58, 0.71)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	12	12
Mean (SD)	71.67 (12.85)	72.08 (22.71)
Median	70.00	60.00
25th, 75th Percentile	62.50, 82.50	55.00, 97.50
Min, Max	50.0, 90.0	45.0, 100.0
Week 26		
n	12	10
Mean (SD)	77.08 (15.73)	75.00 (17.64)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 92.50	60.00, 90.00
Min, Max	60.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	11	10
Mean (SD)	5.91 (13.00)	3.50 (28.09)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 25.00
Min, Max	-10.0, 35.0	-55.0, 45.0
Week 52		
n	13	11
Mean (SD)	76.92 (19.95)	75.91 (18.55)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.5.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 95.00	60.00, 100.00
Min, Max	35.0, 100.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	6.67 (12.85)	5.45 (21.50)
Median	10.00	5.00
25th, 75th Percentile	2.50, 12.50	0.00, 25.00
Min, Max	-25.0, 25.0	-40.0, 40.0
Difference in change from baseline (95%CI)		-1.21 (-16.41, 13.99)
P-value ^b		0.8699
Hedges'g (95% CI) ^c		-0.07 (-0.88, 0.75)
P-value for interaction term, treatment * [Age at Baseline]		0.5789

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	46	47
Mean (SD)	67.64 (21.01)	66.60 (19.42)
Median	70.00	60.00
25th, 75th Percentile	50.00, 90.00	55.00, 80.00
Min, Max	10.0, 100.0	25.0, 100.0
Week 26		
n	46	43
Mean (SD)	70.76 (19.00)	68.34 (19.66)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 80.00	55.00, 85.00
Min, Max	10.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	44	43
Mean (SD)	3.04 (16.45)	2.65 (20.99)
Median	0.00	5.00
25th, 75th Percentile	-7.50, 10.00	-10.00, 20.00
Min, Max	-40.0, 40.0	-75.0, 45.0
Week 52		
n	46	45
Mean (SD)	70.65 (20.65)	65.78 (20.31)
Median	75.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	50.00, 80.00
Min, Max	25.0, 100.0	20.0, 100.0
Change from baseline to Week 52 ^a		
n	44	45
Mean (SD)	1.90 (17.70)	-0.33 (22.09)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 12.50	-10.00, 15.00
Min, Max	-35.0, 45.0	-80.0, 40.0
Difference in change from baseline (95%CI)		-2.24 (-10.68, 6.21)
P-value ^b		0.5999
Hedges'g (95% CI) ^c		-0.11 (-0.53, 0.31)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	13	12
Mean (SD)	63.65 (21.23)	69.58 (21.58)
Median	65.00	62.50
25th, 75th Percentile	60.00, 75.00	55.00, 92.50
Min, Max	12.5, 90.0	40.0, 100.0
Week 26		
n	13	11
Mean (SD)	73.08 (20.06)	71.36 (19.63)
Median	70.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.5.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	50.00, 85.00
Min, Max	35.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	9.42 (11.19)	-0.91 (26.91)
Median	10.00	0.00
25th, 75th Percentile	0.00, 15.00	-15.00, 20.00
Min, Max	0.0, 35.0	-55.0, 30.0
Week 52		
n	13	11
Mean (SD)	67.69 (21.57)	71.82 (17.93)
Median	70.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 80.00	60.00, 85.00
Min, Max	25.0, 100.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	4.04 (14.56)	-0.45 (11.93)
Median	10.00	0.00
25th, 75th Percentile	-5.00, 12.50	-10.00, 5.00
Min, Max	-30.0, 25.0	-20.0, 20.0
Difference in change from baseline (95%CI)		-4.49 (-15.90, 6.92)
P-value ^b		0.4228
Hedges'g (95% CI) ^c		-0.32 (-1.13, 0.49)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.7962

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	10	15
Mean (SD)	63.50 (20.01)	63.33 (15.08)
Median	55.00	60.00
25th, 75th Percentile	50.00, 90.00	55.00, 80.00
Min, Max	45.0, 95.0	40.0, 90.0
Week 26		
n	10	11
Mean (SD)	62.00 (20.58)	70.00 (16.88)
Median	55.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 70.00	45.00, 85.00
Min, Max	40.0, 100.0	45.0, 85.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-1.50 (10.29)	6.82 (26.10)
Median	0.00	20.00
25th, 75th Percentile	-10.00, 5.00	-15.00, 25.00
Min, Max	-20.0, 10.0	-40.0, 35.0
Week 52		
n	9	12
Mean (SD)	69.44 (27.66)	60.42 (18.88)
Median	65.00	57.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 95.00	45.00, 72.50
Min, Max	25.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	9	12
Mean (SD)	3.89 (19.65)	-2.50 (22.00)
Median	5.00	-2.50
25th, 75th Percentile	-5.00, 10.00	-12.50, 7.50
Min, Max	-25.0, 45.0	-50.0, 40.0
Difference in change from baseline (95%CI)		-6.39 (-25.81, 13.03)
P-value ^b		0.4995
Hedges'g (95% CI) ^c		-0.29 (-1.16, 0.58)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	23	17
Mean (SD)	73.26 (16.62)	62.06 (17.95)
Median	75.00	60.00
25th, 75th Percentile	65.00, 90.00	55.00, 65.00
Min, Max	40.0, 100.0	35.0, 100.0
Week 26		
n	22	17
Mean (SD)	77.73 (12.41)	61.69 (19.73)
Median	77.50	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 85.00	50.00, 70.00
Min, Max	45.0, 100.0	25.0, 90.0
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	3.57 (12.36)	-0.37 (25.86)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 10.00	-10.00, 10.00
Min, Max	-15.0, 35.0	-75.0, 45.0
Week 52		
n	23	17
Mean (SD)	73.04 (20.16)	57.65 (17.95)
Median	75.00	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	50.00, 70.00
Min, Max	35.0, 100.0	20.0, 90.0
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-2.05 (15.09)	-4.41 (23.51)
Median	0.00	-5.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-35.0, 25.0	-80.0, 25.0
Difference in change from baseline (95%CI)		-2.37 (-14.92, 10.19)
P-value ^b		0.7048
Hedges'g (95% CI) ^c		-0.12 (-0.75, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	18	22
Mean (SD)	59.38 (25.45)	71.36 (23.05)
Median	58.13	75.00
25th, 75th Percentile	50.00, 80.00	55.00, 90.00
Min, Max	10.0, 100.0	25.0, 100.0
Week 26		
n	19	21
Mean (SD)	67.37 (23.65)	72.38 (20.71)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	55.00, 90.00
Min, Max	10.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	18	21
Mean (SD)	8.40 (20.07)	1.90 (18.06)
Median	5.00	0.00
25th, 75th Percentile	0.00, 22.50	-5.00, 10.00
Min, Max	-40.0, 40.0	-55.0, 30.0
Week 52		
n	19	22
Mean (SD)	64.21 (20.09)	76.36 (18.59)
Median	60.00	77.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 80.00	60.00, 95.00
Min, Max	25.0, 100.0	40.0, 100.0
Change from baseline to Week 52 ^a		
n	18	22
Mean (SD)	5.07 (16.62)	5.00 (16.40)
Median	7.50	0.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 15.00
Min, Max	-30.0, 30.0	-20.0, 40.0
Difference in change from baseline (95%CI)		-0.07 (-10.69, 10.55)
P-value ^b		0.9895
Hedges'g (95% CI) ^c		0.00 (-0.63, 0.62)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	8	5
Mean (SD)	68.75 (19.41)	78.00 (18.91)
Median	70.00	75.00
25th, 75th Percentile	52.50, 87.50	60.00, 95.00
Min, Max	40.0, 90.0	60.0, 100.0
Week 26		
n	8	5
Mean (SD)	74.38 (16.35)	77.00 (16.05)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	67.50, 82.50	75.00, 80.00
Min, Max	45.0, 100.0	55.0, 100.0
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	5.63 (17.00)	-1.00 (18.84)
Median	7.50	0.00
25th, 75th Percentile	2.50, 12.50	-20.00, 15.00
Min, Max	-30.0, 30.0	-20.0, 20.0
Week 52		
n	8	5
Mean (SD)	75.63 (14.25)	73.00 (17.54)
Median	77.50	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 82.50	65.00, 80.00
Min, Max	55.0, 100.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	6.88 (19.81)	-5.00 (22.36)
Median	10.00	0.00
25th, 75th Percentile	-5.00, 22.50	-10.00, 5.00
Min, Max	-30.0, 30.0	-40.0, 20.0
Difference in change from baseline (95%CI)		-11.88 (-37.94, 14.19)
P-value ^b		0.3375
Hedges'g (95% CI) ^c		-0.53 (-1.66, 0.62)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.7786

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	19	19
Mean (SD)	64.41 (22.39)	71.32 (19.06)
Median	60.00	65.00
25th, 75th Percentile	50.00, 90.00	55.00, 90.00
Min, Max	12.5, 100.0	45.0, 100.0
Week 26		
n	19	17
Mean (SD)	68.95 (18.68)	68.53 (21.99)
Median	70.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 85.00	50.00, 85.00
Min, Max	35.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	4.54 (12.03)	-0.59 (28.77)
Median	5.00	0.00
25th, 75th Percentile	0.00, 13.75	-15.00, 25.00
Min, Max	-20.0, 25.0	-75.0, 45.0
Week 52		
n	18	18
Mean (SD)	69.72 (24.64)	67.78 (22.04)
Median	72.50	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.005.006_qs_sum_ovr_ped_care_soc_bhagv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.00, 90.00	55.00, 85.00
Min, Max	25.0, 100.0	20.0, 100.0
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	3.96 (17.62)	-2.50 (25.10)
Median	5.00	0.00
25th, 75th Percentile	-10.00, 12.50	-10.00, 10.00
Min, Max	-25.0, 45.0	-80.0, 25.0
Difference in change from baseline (95%CI)		-6.46 (-21.15, 8.23)
P-value ^b		0.3779
Hedges'g (95% CI) ^c		-0.29 (-0.95, 0.37)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	18	13
Mean (SD)	65.28 (21.38)	63.08 (23.32)
Median	67.50	60.00
25th, 75th Percentile	55.00, 85.00	50.00, 85.00
Min, Max	10.0, 90.0	25.0, 100.0
Week 26		
n	16	11
Mean (SD)	74.38 (20.97)	65.45 (22.41)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 82.50	50.00, 80.00
Min, Max	10.0, 100.0	25.0, 95.0
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	8.75 (16.88)	2.27 (16.64)
Median	0.00	5.00
25th, 75th Percentile	-2.50, 20.00	-10.00, 20.00
Min, Max	-10.0, 40.0	-30.0, 25.0
Week 52		
n	18	12
Mean (SD)	67.78 (21.98)	65.83 (23.34)
Median	70.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 80.00	45.00, 80.00
Min, Max	35.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	18	12
Mean (SD)	2.50 (18.57)	2.92 (25.54)
Median	7.50	-2.50
25th, 75th Percentile	-5.00, 15.00	-10.00, 20.00
Min, Max	-30.0, 30.0	-50.0, 40.0
Difference in change from baseline (95%CI)		0.42 (-16.05, 16.89)
P-value ^b		0.9590
Hedges'g (95% CI) ^c		0.02 (-0.71, 0.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	22	27
Mean (SD)	70.00 (19.82)	66.30 (18.53)
Median	70.00	60.00
25th, 75th Percentile	50.00, 90.00	55.00, 80.00
Min, Max	40.0, 100.0	35.0, 100.0
Week 26		
n	24	26
Mean (SD)	71.04 (18.65)	70.72 (16.96)
Median	77.50	72.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	57.50, 85.00	55.00, 80.00
Min, Max	30.0, 100.0	35.0, 100.0
Change from baseline to Week 26 ^a		
n	22	26
Mean (SD)	1.36 (17.13)	3.41 (19.67)
Median	2.50	9.38
25th, 75th Percentile	-5.00, 10.00	0.00, 15.00
Min, Max	-40.0, 40.0	-55.0, 30.0
Week 52		
n	23	26
Mean (SD)	71.96 (16.70)	66.92 (17.21)
Median	75.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	55.00, 80.00
Min, Max	45.0, 100.0	40.0, 100.0
Change from baseline to Week 52 ^a		
n	21	26
Mean (SD)	0.95 (15.54)	-0.38 (13.85)
Median	0.00	-2.50
25th, 75th Percentile	-5.00, 10.00	-10.00, 10.00
Min, Max	-35.0, 30.0	-20.0, 30.0
Difference in change from baseline (95%CI)		-1.34 (-9.98, 7.31)
P-value ^b		0.7568
Hedges'g (95% CI) ^c		-0.09 (-0.66, 0.49)
P-value for interaction term, treatment * [Baseline AGV]		0.7402

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	40	44
Mean (SD)	66.72 (20.52)	66.93 (18.27)
Median	65.00	60.00
25th, 75th Percentile	50.00, 85.00	55.00, 80.00
Min, Max	12.5, 100.0	25.0, 100.0
Week 26		
n	40	41
Mean (SD)	71.88 (17.23)	69.76 (18.10)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.5.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 82.50	55.00, 85.00
Min, Max	30.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	5.29 (15.39)	3.78 (18.02)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 13.75	-10.00, 15.00
Min, Max	-40.0, 40.0	-35.0, 45.0
Week 52		
n	39	43
Mean (SD)	69.23 (21.96)	67.67 (18.27)
Median	70.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	55.00, 80.00
Min, Max	25.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	38	43
Mean (SD)	1.48 (17.04)	1.28 (18.06)
Median	2.50	0.00
25th, 75th Percentile	-10.00, 12.50	-10.00, 10.00
Min, Max	-35.0, 30.0	-50.0, 40.0
Difference in change from baseline (95%CI)		-0.20 (-8.00, 7.60)
P-value ^b		0.9592
Hedges'g (95% CI) ^c		-0.01 (-0.45, 0.43)

Max, maximum; Min, minimum; SD, standard deviation.

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^b Two-sided p-value.

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Table 14.2.7.1.5.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported PedsQL : Social Functioning Score		
Baseline		
n	19	15
Mean (SD)	66.84 (22.37)	68.00 (24.19)
Median	70.00	60.00
25th, 75th Percentile	50.00, 90.00	60.00, 95.00
Min, Max	10.0, 100.0	25.0, 100.0
Week 26		
n	19	13
Mean (SD)	70.00 (22.97)	66.44 (24.09)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	45.00, 80.00
Min, Max	10.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	18	13
Mean (SD)	2.78 (16.20)	-3.94 (31.98)
Median	0.00	8.75
25th, 75th Percentile	0.00, 10.00	0.00, 20.00
Min, Max	-30.0, 35.0	-75.0, 25.0
Week 52		
n	20	13
Mean (SD)	71.50 (18.43)	64.62 (25.12)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.5.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	40.00, 80.00
Min, Max	40.0, 100.0	20.0, 100.0
Change from baseline to Week 52 ^a		
n	19	13
Mean (SD)	4.21 (17.02)	-5.77 (26.91)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 15.00	-15.00, 15.00
Min, Max	-30.0, 45.0	-80.0, 20.0
Difference in change from baseline (95%CI)		-9.98 (-25.81, 5.85)
P-value ^b		0.2076
Hedges'g (95% CI) ^c		-0.45 (-1.16, 0.27)
P-value for interaction term, treatment * [Ethnicity]		0.2207

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.1.6.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	32	30
Mean (SD)	73.75 (20.36)	73.00 (17.15)
Median	80.00	70.00
25th, 75th Percentile	60.00, 90.00	60.00, 85.00
Min, Max	20.0, 100.0	40.0, 100.0
Week 26		
n	31	27
Mean (SD)	76.45 (22.55)	70.37 (17.54)
Median	85.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Table 14.2.7.1.6.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	55.00, 85.00
Min, Max	20.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	1.67 (13.54)	-2.96 (22.76)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-10.00, 15.00
Min, Max	-30.0, 30.0	-55.0, 35.0
Week 52		
n	32	29
Mean (SD)	71.76 (20.52)	69.83 (18.35)
Median	72.50	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.6.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 87.50	65.00, 80.00
Min, Max	15.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	-2.70 (18.04)	-3.28 (20.67)
Median	-5.00	0.00
25th, 75th Percentile	-13.75, 10.00	-15.00, 10.00
Min, Max	-45.0, 35.0	-55.0, 30.0
Difference in change from baseline (95%CI)		-0.57 (-10.58, 9.43)
P-value ^b		0.9090
Hedges'g (95% CI) ^c		-0.03 (-0.54, 0.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.006.001_qs_sum_ovr_ped_care_sch_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.6.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	27	29
Mean (SD)	77.96 (19.33)	74.48 (19.01)
Median	85.00	80.00
25th, 75th Percentile	60.00, 95.00	65.00, 90.00
Min, Max	35.0, 100.0	25.0, 100.0
Week 26		
n	28	27
Mean (SD)	75.89 (16.28)	75.37 (17.37)
Median	80.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.6.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 90.00	70.00, 90.00
Min, Max	45.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	27	27
Mean (SD)	-1.11 (14.03)	-0.37 (19.01)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-15.00, 10.00
Min, Max	-35.0, 30.0	-40.0, 45.0
Week 52		
n	27	27
Mean (SD)	77.22 (18.15)	74.07 (15.93)
Median	80.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.6.1
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	60.00, 90.00
Min, Max	50.0, 100.0	40.0, 100.0
Change from baseline to Week 52 ^a		
n	26	27
Mean (SD)	-0.96 (16.31)	-1.67 (17.97)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-10.00, 10.00
Min, Max	-40.0, 25.0	-45.0, 40.0
Difference in change from baseline (95%CI)		-0.71 (-10.18, 8.77)
P-value ^b		0.8818
Hedges'g (95% CI) ^c		-0.04 (-0.58, 0.50)
P-value for interaction term, treatment * [Sex]		0.9850

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	23	30
Mean (SD)	72.83 (21.99)	76.17 (16.49)
Median	85.00	80.00
25th, 75th Percentile	55.00, 90.00	65.00, 90.00
Min, Max	20.0, 100.0	45.0, 100.0
Week 26		
n	24	29
Mean (SD)	72.50 (22.55)	75.34 (16.31)
Median	82.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 87.50	70.00, 90.00
Min, Max	20.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	23	29
Mean (SD)	-0.87 (12.03)	0.00 (19.09)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-30.0, 25.0	-50.0, 45.0
Week 52		
n	22	30
Mean (SD)	71.36 (23.56)	72.50 (18.79)
Median	77.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	65.00, 85.00
Min, Max	15.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	21	30
Mean (SD)	-3.57 (15.98)	-3.67 (18.75)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 0.00	-15.00, 5.00
Min, Max	-45.0, 25.0	-55.0, 40.0
Difference in change from baseline (95%CI)		-0.10 (-10.20, 10.01)
P-value ^b		0.9850
Hedges'g (95% CI) ^c		-0.01 (-0.56, 0.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	24	17
Mean (SD)	80.21 (17.60)	67.35 (19.85)
Median	85.00	65.00
25th, 75th Percentile	62.50, 95.00	55.00, 85.00
Min, Max	40.0, 100.0	25.0, 100.0
Week 26		
n	23	15
Mean (SD)	80.22 (16.55)	68.33 (18.19)
Median	80.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 95.00	50.00, 85.00
Min, Max	45.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	-0.87 (13.62)	-0.33 (21.42)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 5.00	-10.00, 15.00
Min, Max	-30.0, 30.0	-40.0, 30.0
Week 52		
n	24	15
Mean (SD)	78.39 (16.47)	74.33 (17.71)
Median	80.63	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	67.50, 95.00	60.00, 90.00
Min, Max	50.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	-1.82 (18.19)	5.67 (18.11)
Median	-2.50	5.00
25th, 75th Percentile	-11.88, 10.00	-5.00, 25.00
Min, Max	-35.0, 35.0	-30.0, 30.0
Difference in change from baseline (95%CI)		7.49 (-4.62, 19.60)
P-value ^b		0.2181
Hedges'g (95% CI) ^c		0.40 (-0.25, 1.05)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	12	12
Mean (SD)	72.08 (19.59)	76.67 (17.88)
Median	72.50	75.00
25th, 75th Percentile	60.00, 85.00	65.00, 92.50
Min, Max	40.0, 100.0	40.0, 100.0
Week 26		
n	12	10
Mean (SD)	75.83 (19.05)	72.50 (20.03)
Median	70.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 97.50	50.00, 85.00
Min, Max	50.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	11	10
Mean (SD)	5.45 (17.10)	-8.50 (25.28)
Median	10.00	0.00
25th, 75th Percentile	-5.00, 15.00	-15.00, 5.00
Min, Max	-35.0, 30.0	-55.0, 25.0
Week 52		
n	13	11
Mean (SD)	71.54 (17.00)	66.82 (11.24)
Median	70.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.2
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	60.00, 75.00
Min, Max	45.0, 100.0	50.0, 90.0
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	0.83 (18.07)	-10.45 (19.68)
Median	5.00	-5.00
25th, 75th Percentile	-7.50, 15.00	-25.00, 0.00
Min, Max	-40.0, 20.0	-50.0, 25.0
Difference in change from baseline (95%CI)		-11.29 (-27.65, 5.08)
P-value ^b		0.1662
Hedges'g (95% CI) ^c		-0.58 (-1.41, 0.27)
P-value for interaction term, treatment * [Age at Baseline]		0.1530

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	46	47
Mean (SD)	77.61 (20.21)	73.72 (17.40)
Median	85.00	75.00
25th, 75th Percentile	60.00, 95.00	55.00, 90.00
Min, Max	20.0, 100.0	40.0, 100.0
Week 26		
n	46	43
Mean (SD)	76.63 (20.66)	72.33 (17.26)
Median	82.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.6.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	55.00, 85.00
Min, Max	20.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	44	43
Mean (SD)	-1.25 (14.23)	-1.28 (19.79)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 7.50	-10.00, 10.00
Min, Max	-35.0, 30.0	-50.0, 45.0
Week 52		
n	46	45
Mean (SD)	75.03 (20.80)	71.00 (17.92)
Median	80.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	60.00, 80.00
Min, Max	15.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	44	45
Mean (SD)	-3.27 (17.70)	-2.44 (19.44)
Median	0.00	0.00
25th, 75th Percentile	-11.88, 5.00	-15.00, 10.00
Min, Max	-45.0, 35.0	-55.0, 40.0
Difference in change from baseline (95%CI)		0.82 (-7.02, 8.66)
P-value ^b		0.8353
Hedges'g (95% CI) ^c		0.04 (-0.37, 0.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.1.6.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	13	12
Mean (SD)	68.85 (17.46)	73.75 (20.79)
Median	65.00	70.00
25th, 75th Percentile	60.00, 80.00	65.00, 90.00
Min, Max	40.0, 100.0	25.0, 100.0
Week 26		
n	13	11
Mean (SD)	74.62 (16.26)	75.00 (18.97)
Median	70.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	60.00, 95.00
Min, Max	55.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	5.77 (10.58)	-3.18 (25.42)
Median	5.00	5.00
25th, 75th Percentile	-5.00, 10.00	-15.00, 10.00
Min, Max	-5.0, 30.0	-55.0, 30.0
Week 52		
n	13	11
Mean (SD)	71.54 (14.34)	75.45 (14.05)
Median	70.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.3
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	60.00, 85.00
Min, Max	50.0, 95.0	60.0, 100.0
Change from baseline to Week 52 ^a		
n	13	11
Mean (SD)	2.69 (14.81)	-2.73 (19.41)
Median	10.00	-5.00
25th, 75th Percentile	-5.00, 15.00	-20.00, 15.00
Min, Max	-30.0, 20.0	-30.0, 30.0
Difference in change from baseline (95%CI)		-5.42 (-19.91, 9.07)
P-value ^b		0.4462
Hedges'g (95% CI) ^c		-0.31 (-1.11, 0.50)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.4612

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	10	15
Mean (SD)	67.00 (23.24)	66.33 (22.16)
Median	72.50	65.00
25th, 75th Percentile	45.00, 85.00	50.00, 90.00
Min, Max	35.0, 95.0	25.0, 100.0
Week 26		
n	10	11
Mean (SD)	66.00 (20.66)	68.64 (15.51)
Median	65.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 80.00	55.00, 75.00
Min, Max	35.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-1.00 (18.07)	-1.36 (26.28)
Median	-2.50	0.00
25th, 75th Percentile	-10.00, 10.00	-15.00, 20.00
Min, Max	-30.0, 30.0	-40.0, 45.0
Week 52		
n	9	12
Mean (SD)	67.36 (21.33)	71.25 (12.08)
Median	60.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 81.25	60.00, 77.50
Min, Max	35.0, 100.0	60.0, 95.0
Change from baseline to Week 52 ^a		
n	9	12
Mean (SD)	-1.53 (20.21)	3.75 (20.90)
Median	-5.00	2.50
25th, 75th Percentile	-13.75, 15.00	-10.00, 20.00
Min, Max	-35.0, 25.0	-30.0, 40.0
Difference in change from baseline (95%CI)		5.28 (-13.75, 24.30)
P-value ^b		0.5683
Hedges'g (95% CI) ^c		0.25 (-0.63, 1.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	23	17
Mean (SD)	79.13 (16.56)	70.88 (17.25)
Median	80.00	65.00
25th, 75th Percentile	65.00, 95.00	55.00, 80.00
Min, Max	45.0, 100.0	45.0, 100.0
Week 26		
n	22	17
Mean (SD)	78.18 (16.73)	68.53 (16.75)
Median	82.50	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	50.00, 80.00
Min, Max	35.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	-2.86 (13.47)	-2.35 (19.85)
Median	0.00	-5.00
25th, 75th Percentile	-10.00, 5.00	-10.00, 10.00
Min, Max	-35.0, 20.0	-50.0, 35.0
Week 52		
n	23	17
Mean (SD)	76.09 (16.78)	65.29 (17.45)
Median	75.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	60.00, 80.00
Min, Max	45.0, 100.0	30.0, 90.0
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-4.09 (16.52)	-5.59 (18.95)
Median	0.00	-5.00
25th, 75th Percentile	-10.00, 10.00	-15.00, 5.00
Min, Max	-45.0, 20.0	-55.0, 25.0
Difference in change from baseline (95%CI)		-1.50 (-13.02, 10.03)
P-value ^b		0.7938
Hedges'g (95% CI) ^c		-0.08 (-0.72, 0.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	18	22
Mean (SD)	74.17 (22.31)	77.95 (14.03)
Median	77.50	77.50
25th, 75th Percentile	60.00, 95.00	70.00, 85.00
Min, Max	20.0, 100.0	50.0, 100.0
Week 26		
n	19	21
Mean (SD)	74.47 (20.68)	76.90 (18.06)
Median	80.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	65.00, 90.00
Min, Max	20.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	18	21
Mean (SD)	1.67 (11.11)	0.00 (17.89)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 5.00
Min, Max	-20.0, 25.0	-55.0, 30.0
Week 52		
n	19	22
Mean (SD)	74.21 (22.81)	76.82 (18.81)
Median	85.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	65.00, 95.00
Min, Max	15.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	18	22
Mean (SD)	1.11 (16.85)	-1.14 (15.96)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 10.00
Min, Max	-30.0, 35.0	-45.0, 30.0
Difference in change from baseline (95%CI)		-2.25 (-12.78, 8.28)
P-value ^b		0.6681
Hedges'g (95% CI) ^c		-0.13 (-0.76, 0.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	8	5
Mean (SD)	80.00 (17.93)	87.00 (13.04)
Median	85.00	85.00
25th, 75th Percentile	62.50, 95.00	80.00, 100.00
Min, Max	55.0, 100.0	70.0, 100.0
Week 26		
n	8	5
Mean (SD)	87.50 (20.00)	80.00 (20.31)
Median	97.50	85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	80.00, 100.00	70.00, 95.00
Min, Max	45.0, 100.0	50.0, 100.0
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	7.50 (13.09)	-7.00 (27.97)
Median	7.50	0.00
25th, 75th Percentile	-2.50, 15.00	-15.00, 5.00
Min, Max	-10.0, 30.0	-50.0, 25.0
Week 52		
n	8	5
Mean (SD)	76.88 (18.11)	74.00 (16.36)
Median	77.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.5
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 92.50	70.00, 80.00
Min, Max	50.0, 100.0	50.0, 95.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-3.13 (18.31)	-13.00 (28.64)
Median	-5.00	-10.00
25th, 75th Percentile	-10.00, 0.00	-30.00, 0.00
Min, Max	-30.0, 35.0	-50.0, 25.0
Difference in change from baseline (95%CI)		-9.88 (-38.25, 18.50)
P-value ^b		0.4599
Hedges'g (95% CI) ^c		-0.41 (-1.53, 0.73)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.7158

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	19	19
Mean (SD)	73.42 (22.18)	77.11 (15.30)
Median	70.00	75.00
25th, 75th Percentile	60.00, 95.00	65.00, 90.00
Min, Max	35.0, 100.0	55.0, 100.0
Week 26		
n	19	17
Mean (SD)	72.63 (21.69)	72.35 (19.21)
Median	70.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 95.00	55.00, 85.00
Min, Max	35.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	-0.79 (15.02)	-3.82 (24.21)
Median	0.00	-5.00
25th, 75th Percentile	-10.00, 10.00	-15.00, 15.00
Min, Max	-30.0, 30.0	-50.0, 45.0
Week 52		
n	18	18
Mean (SD)	72.85 (19.37)	72.50 (16.83)
Median	75.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	60.00, 90.00
Min, Max	35.0, 100.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-1.04 (15.71)	-5.00 (21.14)
Median	-2.50	0.00
25th, 75th Percentile	-10.00, 15.00	-10.00, 5.00
Min, Max	-35.0, 25.0	-55.0, 40.0
Difference in change from baseline (95%CI)		-3.96 (-16.58, 8.66)
P-value ^b		0.5280
Hedges'g (95% CI) ^c		-0.21 (-0.86, 0.45)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	18	13
Mean (SD)	73.61 (22.22)	71.54 (18.75)
Median	80.00	70.00
25th, 75th Percentile	60.00, 90.00	55.00, 90.00
Min, Max	20.0, 100.0	40.0, 95.0
Week 26		
n	16	11
Mean (SD)	78.44 (22.04)	72.73 (19.41)
Median	85.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 95.00	50.00, 90.00
Min, Max	20.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	3.44 (14.91)	0.00 (16.88)
Median	0.00	5.00
25th, 75th Percentile	-2.50, 12.50	-10.00, 10.00
Min, Max	-35.0, 30.0	-40.0, 25.0
Week 52		
n	18	12
Mean (SD)	74.72 (21.86)	70.42 (22.41)
Median	72.50	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 95.00	60.00, 90.00
Min, Max	15.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	18	12
Mean (SD)	1.11 (20.19)	0.42 (19.00)
Median	2.50	2.50
25th, 75th Percentile	-10.00, 10.00	-17.50, 17.50
Min, Max	-40.0, 35.0	-30.0, 25.0
Difference in change from baseline (95%CI)		-0.69 (-15.76, 14.37)
P-value ^b		0.9254
Hedges'g (95% CI) ^c		-0.03 (-0.76, 0.70)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	22	27
Mean (SD)	79.32 (15.61)	72.41 (19.53)
Median	85.00	70.00
25th, 75th Percentile	60.00, 90.00	60.00, 85.00
Min, Max	50.0, 100.0	25.0, 100.0
Week 26		
n	24	26
Mean (SD)	77.50 (16.55)	73.27 (16.12)
Median	82.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	67.50, 87.50	60.00, 85.00
Min, Max	45.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	22	26
Mean (SD)	-0.91 (11.82)	-0.96 (20.59)
Median	-2.50	0.00
25th, 75th Percentile	-10.00, 5.00	-15.00, 10.00
Min, Max	-20.0, 25.0	-55.0, 35.0
Week 52		
n	23	26
Mean (SD)	75.00 (18.46)	72.12 (15.37)
Median	80.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.6
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	65.00, 80.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	21	26
Mean (SD)	-5.24 (15.69)	-2.12 (18.56)
Median	-5.00	-2.50
25th, 75th Percentile	-10.00, 0.00	-15.00, 10.00
Min, Max	-45.0, 25.0	-45.0, 30.0
Difference in change from baseline (95%CI)		3.12 (-7.12, 13.37)
P-value ^b		0.5425
Hedges'g (95% CI) ^c		0.18 (-0.40, 0.75)
P-value for interaction term, treatment * [Baseline AGV]		0.6850

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	40	44
Mean (SD)	76.00 (17.98)	73.52 (15.69)
Median	80.00	70.00
25th, 75th Percentile	60.00, 90.00	65.00, 85.00
Min, Max	40.0, 100.0	40.0, 100.0
Week 26		
n	40	41
Mean (SD)	76.13 (19.03)	72.44 (16.74)
Median	80.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.1.6.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	60.00, 85.00
Min, Max	35.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	0.51 (14.99)	-1.34 (18.20)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-35.0, 30.0	-50.0, 45.0
Week 52		
n	39	43
Mean (SD)	74.36 (19.34)	72.56 (15.83)
Median	75.00	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	65.00, 85.00
Min, Max	35.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	38	43
Mean (SD)	-2.11 (18.91)	-1.05 (16.96)
Median	-2.50	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-45.0, 35.0	-50.0, 40.0
Difference in change from baseline (95%CI)		1.06 (-6.87, 8.99)
P-value ^b		0.7912
Hedges'g (95% CI) ^c		0.06 (-0.38, 0.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported PedsQL : School Functioning Score		
Baseline		
n	19	15
Mean (SD)	75.00 (23.80)	74.33 (24.04)
Median	85.00	80.00
25th, 75th Percentile	60.00, 95.00	55.00, 100.00
Min, Max	20.0, 100.0	25.0, 100.0
Week 26		
n	19	13
Mean (SD)	76.32 (21.46)	74.23 (20.29)
Median	85.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.1.6.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 95.00	55.00, 90.00
Min, Max	20.0, 100.0	45.0, 95.0
Change from baseline to Week 26 ^a		
n	18	13
Mean (SD)	0.00 (10.85)	-2.69 (28.40)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 10.00	-5.00, 15.00
Min, Max	-20.0, 15.0	-55.0, 35.0
Week 52		
n	20	13
Mean (SD)	74.06 (20.32)	69.62 (21.74)
Median	80.63	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.1.6.7
Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 90.00	60.00, 80.00
Min, Max	15.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	19	13
Mean (SD)	-1.51 (13.38)	-7.31 (25.71)
Median	0.00	-5.00
25th, 75th Percentile	-5.00, 10.00	-25.00, 10.00
Min, Max	-30.0, 25.0	-55.0, 30.0
Difference in change from baseline (95%CI)		-5.79 (-22.21, 10.62)
P-value ^b		0.4659
Hedges'g (95% CI) ^c		-0.29 (-1.00, 0.42)
P-value for interaction term, treatment * [Ethnicity]		0.3779

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.001.006.007_qs_sum_ovr_ped_care_sch_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported PedsQL : Total Score		
Baseline		
n	20	15
Mean (SD)	75.94 (15.30)	75.29 (11.77)
Median	81.69	77.38
25th, 75th Percentile	66.31, 88.91	70.45, 83.70
Min, Max	45.7, 94.6	50.0, 93.5
Week 26		
n	19	14
Mean (SD)	74.54 (13.56)	72.71 (11.27)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.001_qs_sum_ovr_ped_self_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.26	72.29
25th, 75th Percentile	66.30, 83.70	64.13, 81.52
Min, Max	43.5, 95.7	53.3, 90.2
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-2.73 (10.48)	-1.98 (6.15)
Median	-1.09	-0.99
25th, 75th Percentile	-9.78, 3.26	-6.52, 3.26
Min, Max	-26.1, 15.2	-13.8, 6.3
Week 52		
n	23	17
Mean (SD)	73.65 (13.33)	73.05 (11.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.91	75.00
25th, 75th Percentile	67.39, 84.78	65.91, 80.43
Min, Max	48.9, 96.7	45.7, 90.2
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	-2.23 (17.31)	-1.52 (13.10)
Median	0.00	-0.11
25th, 75th Percentile	-13.05, 9.78	-8.70, 8.70
Min, Max	-39.8, 22.8	-32.6, 15.9
Difference in change from baseline (95%CI)		0.71 (-10.66, 12.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.001_qs_sum_ovr_ped_self_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8988
Hedges'g (95% CI) ^c		0.04 (-0.65, 0.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.001_qs_sum_ovr_ped_self_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported PedsQL : Total Score		
Baseline		
n	15	13
Mean (SD)	74.49 (15.03)	72.66 (12.31)
Median	72.83	69.57
25th, 75th Percentile	66.30, 93.18	63.04, 84.78
Min, Max	42.4, 96.7	52.2, 91.3
Week 26		
n	20	16
Mean (SD)	74.88 (16.48)	74.39 (10.58)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.001_qs_sum_ovr_ped_self_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.72	73.92
25th, 75th Percentile	62.97, 87.50	67.39, 82.61
Min, Max	44.6, 97.8	56.5, 92.4
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	2.82 (8.85)	2.63 (12.35)
Median	3.26	0.00
25th, 75th Percentile	-2.96, 10.87	-4.89, 8.15
Min, Max	-18.5, 15.2	-14.1, 30.4
Week 52		
n	20	17
Mean (SD)	68.67 (12.76)	78.84 (12.35)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	69.03	81.52
25th, 75th Percentile	60.87, 79.35	70.65, 86.96
Min, Max	47.8, 89.1	55.4, 97.8
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	-3.08 (12.43)	3.86 (14.71)
Median	-1.09	1.09
25th, 75th Percentile	-10.87, 5.43	-5.43, 14.13
Min, Max	-34.5, 18.2	-20.7, 34.8
Difference in change from baseline (95%CI)		6.94 (-4.06, 17.94)
P-value ^b		0.2054

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.50 (-0.30, 1.29)
P-value for interaction term, treatment * [Sex]		0.4305

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported PedsQL : Total Score		
Week 26		
n	5	4
Mean (SD)	62.39 (16.64)	75.00 (15.45)
Median	54.35	75.55
25th, 75th Percentile	53.26, 76.09	63.05, 86.96
Min, Max	44.6, 83.7	56.5, 92.4
Week 52		
n	8	8
Mean (SD)	61.42 (11.52)	80.84 (11.94)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.002_qs_sum_ovr_ped_self_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	63.05	78.26
25th, 75th Percentile	50.00, 69.03	72.83, 91.31
Min, Max	47.8, 79.4	64.1, 97.8
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.002_qs_sum_ovr_ped_self_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported PedsQL : Total Score		
Baseline		
n	23	16
Mean (SD)	73.41 (15.51)	72.69 (9.27)
Median	70.65	70.65
25th, 75th Percentile	64.13, 86.96	65.76, 80.44
Min, Max	42.4, 95.7	57.6, 85.9
Week 26		
n	23	15
Mean (SD)	75.09 (15.68)	70.47 (9.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	76.09	69.57
25th, 75th Percentile	66.30, 86.96	63.04, 79.55
Min, Max	43.5, 97.8	56.8, 85.9
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	-0.32 (10.55)	-1.34 (8.15)
Median	-0.55	0.00
25th, 75th Percentile	-3.27, 8.69	-6.52, 3.26
Min, Max	-26.1, 15.2	-14.1, 17.4
Week 52		
n	23	15
Mean (SD)	71.48 (12.39)	71.67 (14.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.002_qs_sum_ovr_ped_self_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.65	78.26
25th, 75th Percentile	65.22, 81.52	56.52, 83.70
Min, Max	47.8, 91.3	45.7, 90.2
Change from baseline to Week 52 ^a		
n	22	14
Mean (SD)	-2.81 (15.32)	-0.85 (12.10)
Median	0.35	1.53
25th, 75th Percentile	-13.05, 8.70	-6.52, 5.44
Min, Max	-34.5, 21.7	-32.6, 14.1
Difference in change from baseline (95%CI)		1.96 (-7.89, 11.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6887
Hedges'g (95% CI) ^c		0.14 (-0.54, 0.80)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported PedsQL : Total Score		
Baseline		
n	12	12
Mean (SD)	78.98 (13.79)	75.91 (14.92)
Median	79.52	81.52
25th, 75th Percentile	70.66, 90.76	65.22, 87.50
Min, Max	51.1, 96.7	50.0, 93.5
Week 26		
n	11	11
Mean (SD)	79.54 (9.68)	77.37 (10.78)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.43	81.52
25th, 75th Percentile	73.91, 82.61	72.83, 84.78
Min, Max	63.0, 96.7	53.3, 90.2
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	0.29 (9.18)	2.17 (11.39)
Median	1.63	1.09
25th, 75th Percentile	-8.69, 7.60	-6.52, 4.35
Min, Max	-16.3, 12.0	-9.8, 30.4
Week 52		
n	12	11
Mean (SD)	77.66 (12.41)	78.21 (8.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the mean score (sum of all items/number of items answered on all scales).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	79.01	80.43
25th, 75th Percentile	70.66, 86.96	72.83, 85.87
Min, Max	50.0, 96.7	65.2, 87.0
Change from baseline to Week 52 ^a		
n	11	11
Mean (SD)	-2.23 (15.27)	3.01 (16.05)
Median	-1.09	1.09
25th, 75th Percentile	-6.52, 5.44	-8.70, 15.91
Min, Max	-39.8, 22.8	-20.7, 34.8
Difference in change from baseline (95%CI)		5.24 (-8.69, 19.17)
P-value ^b		0.4419

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.001.002_qs_sum_ovr_ped_self_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.32 (-0.52, 1.16)
P-value for interaction term, treatment * [Age at Baseline]		0.6854

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported PedsQL : Total Score		
Baseline		
n	22	16
Mean (SD)	76.31 (14.09)	71.06 (11.06)
Median	76.09	70.55
25th, 75th Percentile	69.57, 88.04	62.50, 80.44
Min, Max	45.7, 96.7	50.0, 89.1
Week 26		
n	27	19
Mean (SD)	73.79 (15.46)	71.14 (11.40)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	76.09	69.57
25th, 75th Percentile	65.22, 84.78	63.04, 81.52
Min, Max	43.5, 97.8	53.3, 92.4
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	-0.97 (11.08)	-0.11 (7.50)
Median	0.00	1.29
25th, 75th Percentile	-7.07, 8.15	-6.52, 3.26
Min, Max	-26.1, 15.2	-13.8, 17.4
Week 52		
n	31	23
Mean (SD)	70.58 (12.56)	74.79 (13.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.65	78.26
25th, 75th Percentile	65.22, 79.35	64.13, 83.70
Min, Max	47.8, 91.3	45.7, 97.8
Change from baseline to Week 52 ^a		
n	21	14
Mean (SD)	-3.39 (15.23)	0.75 (13.32)
Median	0.00	2.07
25th, 75th Percentile	-10.87, 6.52	-7.61, 13.04
Min, Max	-39.8, 21.7	-32.6, 15.9
Difference in change from baseline (95%CI)		4.14 (-6.05, 14.32)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.4143
Hedges'g (95% CI) ^c		0.28 (-0.40, 0.96)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported PedsQL : Total Score		
Baseline		
n	13	12
Mean (SD)	73.64 (16.83)	78.08 (12.19)
Median	72.83	81.52
25th, 75th Percentile	63.04, 91.30	70.11, 85.87
Min, Max	42.4, 95.7	52.2, 93.5
Week 26		
n	12	11
Mean (SD)	76.80 (14.08)	77.87 (8.37)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.43	81.52
25th, 75th Percentile	69.57, 86.42	70.65, 84.78
Min, Max	44.6, 92.4	63.0, 90.2
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	1.28 (8.13)	0.50 (12.31)
Median	1.09	-3.26
25th, 75th Percentile	-5.37, 7.44	-6.52, 4.35
Min, Max	-9.8, 15.2	-14.1, 30.4
Week 52		
n	12	11
Mean (SD)	73.26 (15.00)	78.35 (8.77)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	76.09	79.35
25th, 75th Percentile	63.05, 83.94	70.65, 86.96
Min, Max	47.8, 96.7	65.2, 90.2
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	-1.26 (15.34)	0.98 (15.04)
Median	-2.14	-3.26
25th, 75th Percentile	-9.78, 7.07	-6.68, 5.44
Min, Max	-34.5, 22.8	-20.7, 34.8
Difference in change from baseline (95%CI)		2.24 (-10.96, 15.43)
P-value ^b		0.7278

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.14 (-0.68, 0.96)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.8133

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported PedsQL : Total Score		
Baseline		
n	4	8
Mean (SD)	65.49 (6.24)	76.36 (9.96)
Median	67.39	76.09
25th, 75th Percentile	61.41, 69.57	68.48, 84.79
Min, Max	56.5, 70.7	63.0, 89.1
Week 26		
n	6	7
Mean (SD)	57.61 (15.09)	74.69 (7.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	52.72	69.57
25th, 75th Percentile	44.57, 71.74	68.48, 81.52
Min, Max	43.5, 80.4	66.3, 85.9
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-3.53 (14.54)	1.99 (11.97)
Median	-3.80	1.63
25th, 75th Percentile	-15.76, 8.70	-7.61, 13.04
Min, Max	-18.5, 12.0	-14.1, 17.4
Week 52		
n	6	9
Mean (SD)	67.94 (9.19)	78.38 (11.20)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	69.02	79.35
25th, 75th Percentile	67.39, 72.83	75.00, 83.70
Min, Max	51.1, 78.3	55.4, 95.7
Change from baseline to Week 52 ^a		
n	4	5
Mean (SD)	6.80 (10.21)	2.61 (12.76)
Median	3.27	-3.26
25th, 75th Percentile	0.55, 13.05	-7.61, 14.13
Min, Max	-1.1, 21.7	-8.7, 18.5
Difference in change from baseline (95%CI)		-4.19 (-22.80, 14.43)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6113
Hedges'g (95% CI) ^c		-0.32 (-1.63, 1.02)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported PedsQL : Total Score		
Baseline		
n	14	8
Mean (SD)	77.19 (15.07)	69.18 (11.60)
Median	83.33	71.20
25th, 75th Percentile	63.04, 89.77	60.33, 77.82
Min, Max	51.1, 96.7	50.0, 84.8
Week 26		
n	13	8
Mean (SD)	80.77 (12.16)	68.54 (12.30)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.52	67.94
25th, 75th Percentile	76.09, 86.96	57.22, 80.54
Min, Max	54.4, 97.8	53.3, 83.7
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	0.92 (8.79)	-0.64 (6.00)
Median	0.00	0.55
25th, 75th Percentile	-3.26, 7.60	-1.63, 2.28
Min, Max	-16.3, 15.2	-13.8, 6.3
Week 52		
n	16	9
Mean (SD)	70.27 (15.02)	66.74 (13.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.28	65.91
25th, 75th Percentile	56.52, 82.61	58.70, 72.83
Min, Max	47.8, 90.2	45.7, 90.2
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-4.09 (16.27)	-2.11 (14.81)
Median	-3.19	1.53
25th, 75th Percentile	-13.04, 5.43	-6.52, 4.90
Min, Max	-39.8, 22.8	-32.6, 15.9
Difference in change from baseline (95%CI)		1.98 (-12.83, 16.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.7829
Hedges'g (95% CI) ^c		0.12 (-0.76, 1.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported PedsQL : Total Score		
Baseline		
n	11	8
Mean (SD)	74.21 (14.59)	72.94 (12.82)
Median	73.91	73.91
25th, 75th Percentile	68.18, 86.96	64.58, 81.52
Min, Max	42.4, 94.1	52.2, 91.3
Week 26		
n	14	11
Mean (SD)	75.15 (12.56)	75.00 (11.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.18	72.83
25th, 75th Percentile	66.30, 83.70	64.13, 84.78
Min, Max	44.6, 92.4	56.5, 92.4
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	0.98 (7.33)	1.39 (12.95)
Median	0.00	-2.62
25th, 75th Percentile	-3.27, 8.69	-6.52, 4.35
Min, Max	-10.9, 12.0	-9.8, 30.4
Week 52		
n	16	12
Mean (SD)	69.73 (11.89)	80.24 (10.40)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	69.03	82.61
25th, 75th Percentile	61.96, 78.26	74.38, 86.42
Min, Max	50.0, 89.1	56.5, 97.8
Change from baseline to Week 52 ^a		
n	11	8
Mean (SD)	-4.40 (15.39)	4.76 (14.17)
Median	-2.18	-0.01
25th, 75th Percentile	-7.61, 7.60	-5.98, 10.98
Min, Max	-34.5, 18.2	-6.7, 34.8
Difference in change from baseline (95%CI)		9.16 (-5.45, 23.77)

Max, maximum; Min, minimum; SD, standard deviation.

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^b Two-sided p-value.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2033
Hedges'g (95% CI) ^c		0.59 (-0.35, 1.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported PedsQL : Total Score		
Baseline		
n	6	4
Mean (SD)	79.53 (19.35)	81.52 (13.60)
Median	85.87	85.33
25th, 75th Percentile	69.57, 94.57	73.37, 89.68
Min, Max	45.7, 95.7	62.0, 93.5
Week 26		
n	6	4
Mean (SD)	77.72 (15.60)	77.99 (11.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.72	78.81
25th, 75th Percentile	68.48, 92.39	69.57, 86.42
Min, Max	54.4, 95.7	64.1, 90.2
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	-1.81 (14.60)	-3.53 (4.89)
Median	0.00	-3.26
25th, 75th Percentile	-8.69, 8.70	-6.52, -0.55
Min, Max	-26.1, 15.2	-9.8, 2.2
Week 52		
n	5	4
Mean (SD)	83.91 (10.27)	78.26 (10.69)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.52	78.81
25th, 75th Percentile	79.35, 91.30	70.11, 86.42
Min, Max	70.7, 96.7	65.2, 90.2
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	-2.39 (15.71)	-3.26 (15.29)
Median	5.44	-2.72
25th, 75th Percentile	-14.13, 9.78	-15.76, 9.24
Min, Max	-23.9, 10.9	-20.7, 13.0
Difference in change from baseline (95%CI)		-0.87 (-25.50, 23.77)
P-value ^b		0.9359

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.05 (-1.36, 1.27)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.6892

Max, maximum; Min, minimum; SD, standard deviation.

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^b Two-sided p-value.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported PedsQL : Total Score		
Baseline		
n	13	9
Mean (SD)	72.13 (14.81)	70.17 (12.68)
Median	68.48	70.45
25th, 75th Percentile	64.13, 83.70	61.96, 82.61
Min, Max	42.4, 94.6	50.0, 84.8
Week 26		
n	14	10
Mean (SD)	69.24 (14.13)	71.31 (12.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.92	70.66
25th, 75th Percentile	60.71, 80.43	64.13, 81.52
Min, Max	43.5, 84.8	53.3, 92.4
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-1.66 (10.63)	0.41 (4.96)
Median	0.00	1.73
25th, 75th Percentile	-7.47, 5.44	-1.63, 3.26
Min, Max	-26.1, 12.0	-9.8, 6.3
Week 52		
n	14	11
Mean (SD)	68.51 (12.25)	76.05 (12.97)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	69.02	75.00
25th, 75th Percentile	65.22, 78.26	65.91, 84.78
Min, Max	48.9, 88.0	55.4, 97.8
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-2.65 (16.15)	5.66 (7.93)
Median	0.00	3.81
25th, 75th Percentile	-17.40, 7.07	1.53, 13.15
Min, Max	-28.3, 21.7	-7.6, 15.9
Difference in change from baseline (95%CI)		8.31 (-4.69, 21.31)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.6
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.1961
Hedges'g (95% CI) ^c		0.59 (-0.34, 1.49)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported PedsQL : Total Score		
Baseline		
n	14	8
Mean (SD)	76.39 (15.28)	77.85 (9.38)
Median	75.00	74.46
25th, 75th Percentile	69.57, 90.22	70.11, 86.96
Min, Max	51.1, 96.7	68.5, 91.3
Week 26		
n	12	10
Mean (SD)	78.71 (11.60)	73.53 (11.14)

Max, maximum; Min, minimum; SD, standard deviation.

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Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.81	77.28
25th, 75th Percentile	73.37, 86.42	64.13, 81.52
Min, Max	54.4, 96.7	56.5, 85.9
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	1.25 (8.86)	-3.20 (9.65)
Median	3.26	-6.52
25th, 75th Percentile	-3.27, 7.61	-8.70, 0.65
Min, Max	-16.3, 15.2	-13.8, 17.4
Week 52		
n	15	9
Mean (SD)	73.07 (14.92)	76.69 (15.15)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.17	81.52
25th, 75th Percentile	67.39, 81.52	79.35, 85.87
Min, Max	47.8, 96.7	45.7, 90.2
Change from baseline to Week 52 ^a		
n	14	7
Mean (SD)	-1.52 (15.46)	-4.66 (15.92)
Median	-1.09	-5.43
25th, 75th Percentile	-10.87, 7.60	-14.13, 8.70
Min, Max	-39.8, 22.8	-32.6, 14.1
Difference in change from baseline (95%CI)		-3.14 (-18.26, 11.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6687
Hedges'g (95% CI) ^c		-0.19 (-1.10, 0.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the mean score (sum of all items/number of items answered on all scales).

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported PedsQL : Total Score		
Baseline		
n	8	11
Mean (SD)	78.62 (15.70)	74.51 (12.85)
Median	82.26	77.17
25th, 75th Percentile	72.28, 90.07	63.04, 85.87
Min, Max	45.7, 94.1	52.2, 93.5
Week 26		
n	13	10
Mean (SD)	76.92 (17.61)	75.98 (9.59)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	83.70	77.18
25th, 75th Percentile	65.22, 90.22	68.48, 82.61
Min, Max	44.6, 97.8	63.0, 90.2
Change from baseline to Week 26 ^a		
n	8	10
Mean (SD)	0.46 (11.36)	2.61 (12.16)
Median	0.61	0.55
25th, 75th Percentile	-6.92, 9.79	-3.26, 4.35
Min, Max	-18.5, 15.2	-14.1, 30.4
Week 52		
n	14	14
Mean (SD)	72.28 (12.49)	75.38 (10.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.66	74.38
25th, 75th Percentile	63.04, 84.78	67.39, 82.61
Min, Max	51.1, 91.3	56.5, 95.7
Change from baseline to Week 52 ^a		
n	7	10
Mean (SD)	-4.75 (14.35)	0.86 (15.68)
Median	0.00	-2.72
25th, 75th Percentile	-7.61, 2.18	-6.68, 4.35
Min, Max	-34.5, 10.9	-20.7, 34.8
Difference in change from baseline (95%CI)		5.61 (-10.32, 21.53)
P-value ^b		0.4645

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.35 (-0.63, 1.32)
P-value for interaction term, treatment * [Baseline AGV]		0.4704

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported PedsQL : Total Score		
Baseline		
n	24	22
Mean (SD)	73.94 (14.06)	72.78 (12.65)
Median	73.37	71.20
25th, 75th Percentile	68.33, 85.33	63.04, 83.70
Min, Max	42.4, 96.7	50.0, 93.5
Week 26		
n	27	25
Mean (SD)	75.56 (13.69)	73.19 (11.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.26	72.83
25th, 75th Percentile	66.30, 83.70	64.13, 81.52
Min, Max	44.6, 97.8	53.3, 92.4
Change from baseline to Week 26 ^a		
n	22	21
Mean (SD)	1.37 (9.41)	0.59 (9.06)
Median	2.72	0.00
25th, 75th Percentile	-3.27, 8.69	-3.26, 3.26
Min, Max	-18.5, 15.2	-13.8, 30.4
Week 52		
n	30	27
Mean (SD)	72.13 (11.90)	74.87 (12.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	71.20	75.00
25th, 75th Percentile	66.30, 79.76	65.91, 84.78
Min, Max	48.9, 91.3	45.7, 97.8
Change from baseline to Week 52 ^a		
n	23	21
Mean (SD)	-1.46 (14.26)	0.81 (14.86)
Median	0.00	1.09
25th, 75th Percentile	-7.61, 7.60	-7.61, 13.04
Min, Max	-39.8, 22.8	-32.6, 34.8
Difference in change from baseline (95%CI)		2.27 (-6.59, 11.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6084
Hedges'g (95% CI) ^c		0.15 (-0.44, 0.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported PedsQL : Total Score		
Baseline		
n	11	6
Mean (SD)	78.32 (17.14)	78.80 (7.43)
Median	84.09	81.52
25th, 75th Percentile	60.87, 94.05	70.65, 84.78
Min, Max	52.2, 95.7	68.5, 85.9
Week 26		
n	12	5
Mean (SD)	72.83 (17.92)	75.65 (9.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.72	75.00
25th, 75th Percentile	58.70, 88.59	68.48, 84.78
Min, Max	43.5, 92.4	64.1, 85.9
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	-3.44 (10.96)	-1.74 (12.69)
Median	-2.31	-6.52
25th, 75th Percentile	-8.69, 2.87	-9.78, 4.35
Min, Max	-26.1, 15.2	-14.1, 17.4
Week 52		
n	13	7
Mean (SD)	69.48 (16.06)	80.10 (10.36)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	69.57	79.35
25th, 75th Percentile	58.70, 81.52	73.75, 90.22
Min, Max	47.8, 96.7	64.1, 95.7
Change from baseline to Week 52 ^a		
n	10	4
Mean (SD)	-5.28 (17.28)	1.05 (7.21)
Median	-5.72	1.09
25th, 75th Percentile	-14.13, 5.44	-4.97, 7.07
Min, Max	-34.5, 21.7	-6.7, 8.7
Difference in change from baseline (95%CI)		6.33 (-13.51, 26.17)
P-value ^b		0.5000

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.1.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.39 (-0.79, 1.55)
P-value for interaction term, treatment * [Ethnicity]		0.6795

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	20	15
Mean (SD)	79.80 (16.97)	81.30 (11.48)
Median	84.38	84.38
25th, 75th Percentile	67.19, 93.75	71.88, 90.63
Min, Max	40.6, 96.9	56.3, 93.8
Week 26		
n	19	14
Mean (SD)	76.65 (16.55)	77.68 (13.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	76.57
25th, 75th Percentile	71.88, 90.63	65.63, 87.50
Min, Max	37.5, 93.8	56.3, 100.0
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-3.68 (12.46)	-2.96 (11.10)
Median	-3.12	0.00
25th, 75th Percentile	-15.63, 6.25	-9.38, 6.25
Min, Max	-21.9, 15.6	-25.0, 13.8
Week 52		
n	23	17
Mean (SD)	77.09 (14.53)	75.24 (13.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	78.13
25th, 75th Percentile	65.63, 87.50	68.75, 85.71
Min, Max	53.1, 100.0	46.4, 93.8
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	-1.98 (18.67)	-3.12 (14.41)
Median	-1.56	-3.13
25th, 75th Percentile	-12.50, 12.50	-9.82, 4.17
Min, Max	-36.6, 31.3	-37.5, 23.2
Difference in change from baseline (95%CI)		-1.14 (-13.48, 11.20)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8518
Hedges'g (95% CI) ^c		-0.07 (-0.76, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	15	13
Mean (SD)	73.33 (18.61)	72.84 (15.90)
Median	78.13	75.00
25th, 75th Percentile	62.50, 84.38	59.38, 84.38
Min, Max	31.3, 100.0	50.0, 96.9
Week 26		
n	20	16
Mean (SD)	71.03 (21.83)	75.20 (13.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.44	78.13
25th, 75th Percentile	60.27, 87.51	67.19, 82.82
Min, Max	31.3, 100.0	43.8, 96.9
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	1.59 (11.22)	1.30 (13.55)
Median	3.13	3.12
25th, 75th Percentile	-6.25, 9.37	-6.25, 10.94
Min, Max	-18.8, 18.8	-25.0, 25.0
Week 52		
n	20	17
Mean (SD)	65.94 (17.26)	79.78 (15.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	67.19	84.38
25th, 75th Percentile	51.57, 76.57	71.88, 90.63
Min, Max	37.5, 96.9	46.9, 100.0
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	-2.07 (13.48)	3.41 (13.29)
Median	1.05	3.12
25th, 75th Percentile	-12.50, 9.37	-9.37, 6.25
Min, Max	-28.1, 18.8	-12.5, 31.3
Difference in change from baseline (95%CI)		5.48 (-5.50, 16.46)
P-value ^b		0.3130

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.40 (-0.39, 1.18)
P-value for interaction term, treatment * [Sex]		0.4261

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported PedsQL : Physical Health Summary Score		
Week 26		
n	5	4
Mean (SD)	52.50 (21.12)	80.47 (12.85)
Median	40.63	79.69
25th, 75th Percentile	37.50, 68.75	71.88, 89.07
Min, Max	34.4, 81.3	65.6, 96.9
Week 52		
n	8	8
Mean (SD)	58.60 (12.47)	80.86 (14.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	57.82	81.25
25th, 75th Percentile	51.57, 68.76	68.76, 93.76
Min, Max	37.5, 75.0	59.4, 100.0
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	23	16
Mean (SD)	74.45 (19.47)	74.85 (13.47)
Median	78.13	75.01
25th, 75th Percentile	62.50, 93.75	63.40, 85.94
Min, Max	31.3, 96.9	50.0, 93.8
Week 26		
n	23	15
Mean (SD)	76.44 (19.25)	69.79 (12.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	71.88
25th, 75th Percentile	68.75, 90.63	62.50, 78.13
Min, Max	31.3, 100.0	43.8, 90.6
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	-0.48 (13.20)	-4.63 (11.71)
Median	1.57	-6.25
25th, 75th Percentile	-6.25, 9.37	-12.50, 3.13
Min, Max	-21.9, 18.8	-25.0, 13.8
Week 52		
n	23	15
Mean (SD)	73.10 (17.49)	74.38 (16.20)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	78.13
25th, 75th Percentile	56.25, 87.50	62.50, 87.50
Min, Max	37.5, 100.0	46.9, 96.9
Change from baseline to Week 52 ^a		
n	22	14
Mean (SD)	-1.27 (17.04)	-0.05 (14.40)
Median	0.53	3.13
25th, 75th Percentile	-12.50, 9.38	-9.37, 6.25
Min, Max	-31.3, 31.3	-37.5, 23.2
Difference in change from baseline (95%CI)		1.22 (-9.95, 12.39)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8259
Hedges'g (95% CI) ^c		0.07 (-0.60, 0.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	12	12
Mean (SD)	81.96 (13.13)	80.73 (14.83)
Median	81.26	85.94
25th, 75th Percentile	71.88, 93.31	68.75, 92.19
Min, Max	62.5, 100.0	56.3, 96.9
Week 26		
n	11	11
Mean (SD)	77.84 (13.22)	83.81 (10.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	84.38
25th, 75th Percentile	68.75, 93.75	78.13, 90.63
Min, Max	56.3, 96.9	62.5, 100.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-2.81 (9.25)	3.98 (11.62)
Median	-1.56	6.25
25th, 75th Percentile	-6.25, 6.25	-6.25, 9.38
Min, Max	-18.8, 9.4	-18.8, 25.0
Week 52		
n	12	11
Mean (SD)	78.48 (12.95)	79.34 (12.77)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.65	81.25
25th, 75th Percentile	67.19, 90.63	75.00, 85.71
Min, Max	56.3, 100.0	46.4, 96.9
Change from baseline to Week 52 ^a		
n	11	11
Mean (SD)	-3.52 (15.28)	-0.49 (14.26)
Median	0.00	-3.12
25th, 75th Percentile	-9.38, 3.13	-9.82, 0.00
Min, Max	-36.6, 18.8	-12.5, 31.3
Difference in change from baseline (95%CI)		3.03 (-10.11, 16.18)
P-value ^b		0.6358

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.20 (-0.64, 1.03)
P-value for interaction term, treatment * [Age at Baseline]		0.8326

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	22	16
Mean (SD)	78.18 (18.17)	75.64 (14.12)
Median	79.69	75.01
25th, 75th Percentile	62.50, 93.75	63.40, 89.07
Min, Max	40.6, 100.0	50.0, 93.8
Week 26		
n	27	19
Mean (SD)	73.50 (20.84)	73.69 (13.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Physical health summary score is the mean score (sum of items scored/number of items answered in physical functioning domain).

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	71.88
25th, 75th Percentile	56.25, 90.63	62.50, 81.25
Min, Max	31.3, 100.0	43.8, 96.9
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	-0.89 (13.43)	-2.76 (10.98)
Median	2.09	0.00
25th, 75th Percentile	-12.50, 9.37	-6.25, 4.17
Min, Max	-21.9, 18.8	-25.0, 13.8
Week 52		
n	31	23
Mean (SD)	71.27 (16.89)	75.58 (16.27)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	71.88	78.13
25th, 75th Percentile	56.25, 81.25	62.50, 87.50
Min, Max	37.5, 100.0	46.4, 100.0
Change from baseline to Week 52 ^a		
n	21	14
Mean (SD)	-1.99 (18.07)	-2.00 (14.44)
Median	1.05	0.00
25th, 75th Percentile	-12.50, 9.38	-9.82, 4.17
Min, Max	-36.6, 31.3	-37.5, 23.2
Difference in change from baseline (95%CI)		-0.01 (-11.76, 11.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9990
Hedges'g (95% CI) ^c		0.00 (-0.68, 0.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	13	12
Mean (SD)	75.07 (17.48)	79.69 (14.38)
Median	78.13	82.82
25th, 75th Percentile	65.63, 84.38	68.75, 92.19
Min, Max	31.3, 96.9	56.3, 96.9
Week 26		
n	12	11
Mean (SD)	74.37 (16.49)	80.97 (11.04)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	81.25
25th, 75th Percentile	66.52, 85.94	75.00, 87.50
Min, Max	34.4, 93.8	59.4, 100.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	-1.75 (9.69)	1.42 (13.93)
Median	-1.57	3.13
25th, 75th Percentile	-6.25, 4.69	-9.38, 9.38
Min, Max	-17.9, 15.6	-25.0, 25.0
Week 52		
n	12	11
Mean (SD)	73.53 (16.59)	81.54 (10.31)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.09	81.25
25th, 75th Percentile	67.19, 87.50	75.00, 87.50
Min, Max	37.5, 90.6	62.5, 96.9
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	-2.07 (13.28)	1.99 (13.86)
Median	-1.56	0.00
25th, 75th Percentile	-9.38, 4.25	-9.38, 6.25
Min, Max	-28.1, 18.8	-12.5, 31.3
Difference in change from baseline (95%CI)		4.06 (-7.71, 15.83)
P-value ^b		0.4808

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.29 (-0.54, 1.11)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.6316

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	4	8
Mean (SD)	59.38 (6.75)	77.35 (14.15)
Median	60.94	79.69
25th, 75th Percentile	54.69, 64.07	62.50, 90.63
Min, Max	50.0, 65.6	59.4, 93.8
Week 26		
n	6	7
Mean (SD)	47.40 (17.05)	72.77 (6.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	40.63	71.88
25th, 75th Percentile	34.38, 68.75	68.75, 78.13
Min, Max	31.3, 68.8	62.5, 81.3
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-6.25 (16.34)	-3.13 (16.54)
Median	-6.25	-1.57
25th, 75th Percentile	-20.32, 7.81	-18.75, 12.50
Min, Max	-21.9, 9.4	-25.0, 15.6
Week 52		
n	6	9
Mean (SD)	68.23 (16.23)	75.00 (14.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	67.19	78.13
25th, 75th Percentile	56.25, 71.88	65.63, 84.38
Min, Max	50.0, 96.9	46.9, 96.9
Change from baseline to Week 52 ^a		
n	4	5
Mean (SD)	12.50 (12.76)	-1.87 (14.76)
Median	7.81	-9.37
25th, 75th Percentile	4.69, 20.31	-12.50, 3.12
Min, Max	3.1, 31.3	-12.5, 21.9
Difference in change from baseline (95%CI)		-14.37 (-36.48, 7.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.1680
Hedges'g (95% CI) ^c		-0.92 (-2.29, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	14	8
Mean (SD)	81.41 (17.18)	76.04 (16.03)
Median	87.51	79.17
25th, 75th Percentile	68.75, 93.75	64.07, 89.07
Min, Max	40.6, 100.0	50.0, 93.8
Week 26		
n	13	8
Mean (SD)	82.45 (15.96)	74.61 (18.33)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	87.50	78.13
25th, 75th Percentile	78.13, 93.75	60.94, 89.07
Min, Max	40.6, 96.9	43.8, 96.9
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	-0.85 (9.07)	-1.43 (10.51)
Median	-3.12	1.57
25th, 75th Percentile	-6.25, 6.25	-3.13, 3.65
Min, Max	-18.8, 15.6	-25.0, 9.4
Week 52		
n	16	9
Mean (SD)	69.40 (20.10)	71.33 (15.91)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	76.57	71.88
25th, 75th Percentile	53.13, 84.38	59.38, 85.71
Min, Max	37.5, 100.0	50.0, 93.8
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-7.30 (17.63)	-3.22 (14.70)
Median	-5.21	3.13
25th, 75th Percentile	-21.87, 3.12	-5.58, 5.21
Min, Max	-36.6, 18.8	-37.5, 6.3
Difference in change from baseline (95%CI)		4.09 (-11.54, 19.71)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.5905
Hedges'g (95% CI) ^c		0.24 (-0.65, 1.12)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	11	8
Mean (SD)	76.41 (17.44)	76.79 (13.21)
Median	78.13	76.57
25th, 75th Percentile	75.00, 84.38	68.09, 85.94
Min, Max	31.3, 95.8	56.3, 96.9
Week 26		
n	14	11
Mean (SD)	75.80 (16.85)	77.84 (11.90)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	78.13
25th, 75th Percentile	68.75, 81.25	65.63, 87.50
Min, Max	34.4, 100.0	56.3, 96.9
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	1.31 (13.16)	0.17 (13.65)
Median	3.13	-6.25
25th, 75th Percentile	-6.25, 15.62	-7.82, 10.05
Min, Max	-21.9, 18.8	-15.6, 25.0
Week 52		
n	16	12
Mean (SD)	70.71 (12.70)	84.90 (9.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.75	87.50
25th, 75th Percentile	64.07, 81.25	78.13, 89.07
Min, Max	50.0, 96.9	68.8, 100.0
Change from baseline to Week 52 ^a		
n	11	8
Mean (SD)	-2.83 (12.43)	6.81 (14.62)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 6.25	-3.13, 19.42
Min, Max	-21.9, 18.8	-9.4, 31.3
Difference in change from baseline (95%CI)		9.63 (-3.48, 22.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.7.2.2.5
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.1395
Hedges'g (95% CI) ^c		0.69 (-0.26, 1.62)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	6	4
Mean (SD)	79.69 (20.52)	81.25 (17.12)
Median	87.51	87.51
25th, 75th Percentile	62.50, 96.88	70.32, 92.19
Min, Max	46.9, 96.9	56.3, 93.8
Week 26		
n	6	4
Mean (SD)	76.57 (12.92)	82.03 (15.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	82.82
25th, 75th Percentile	71.88, 90.63	70.32, 93.75
Min, Max	56.3, 90.6	62.5, 100.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	-3.13 (13.55)	0.78 (8.98)
Median	0.00	4.69
25th, 75th Percentile	-15.63, 9.37	-4.69, 6.25
Min, Max	-21.9, 9.4	-12.5, 6.3
Week 52		
n	5	4
Mean (SD)	88.13 (8.95)	74.89 (21.09)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	87.50	78.13
25th, 75th Percentile	87.50, 90.63	60.72, 89.07
Min, Max	75.0, 100.0	46.4, 96.9
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	1.87 (18.83)	-6.36 (8.52)
Median	0.00	-9.60
25th, 75th Percentile	-9.38, 15.62	-11.16, -1.57
Min, Max	-21.9, 25.0	-12.5, 6.3
Difference in change from baseline (95%CI)		-8.23 (-32.48, 16.02)
P-value ^b		0.4484

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.48 (-1.80, 0.87)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.1929

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	13	9
Mean (SD)	74.11 (18.71)	75.08 (17.32)
Median	75.00	83.33
25th, 75th Percentile	62.50, 90.63	59.38, 90.63
Min, Max	31.3, 96.9	50.0, 93.8
Week 26		
n	14	10
Mean (SD)	69.77 (19.89)	78.13 (17.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	71.88	81.25
25th, 75th Percentile	62.50, 87.50	62.50, 90.63
Min, Max	34.4, 93.8	43.8, 96.9
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-1.86 (13.07)	2.25 (6.60)
Median	0.00	3.13
25th, 75th Percentile	-6.25, 6.25	-3.13, 5.21
Min, Max	-21.9, 18.8	-6.3, 13.8
Week 52		
n	14	11
Mean (SD)	72.10 (13.84)	75.08 (19.14)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	71.88	84.38
25th, 75th Percentile	65.63, 81.25	53.13, 87.50
Min, Max	50.0, 96.9	46.4, 100.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-0.86 (18.00)	0.02 (11.36)
Median	1.57	0.00
25th, 75th Percentile	-15.63, 9.38	-8.93, 3.65
Min, Max	-31.3, 31.3	-12.5, 23.2
Difference in change from baseline (95%CI)		0.87 (-14.23, 15.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9045
Hedges'g (95% CI) ^c		0.05 (-0.84, 0.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	14	8
Mean (SD)	79.85 (17.23)	81.25 (11.93)
Median	81.26	82.82
25th, 75th Percentile	65.63, 93.75	71.88, 90.63
Min, Max	40.6, 100.0	62.5, 96.9
Week 26		
n	12	10
Mean (SD)	78.13 (14.60)	73.13 (8.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	79.69	71.88
25th, 75th Percentile	71.88, 87.51	65.63, 78.13
Min, Max	40.6, 96.9	62.5, 90.6
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	-2.27 (12.66)	-7.81 (11.45)
Median	-3.12	-6.25
25th, 75th Percentile	-15.63, 9.37	-15.63, -3.13
Min, Max	-21.9, 15.6	-25.0, 12.5
Week 52		
n	15	9
Mean (SD)	74.03 (19.34)	82.99 (14.25)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	87.50
25th, 75th Percentile	56.25, 87.50	78.13, 90.63
Min, Max	37.5, 100.0	50.0, 96.9
Change from baseline to Week 52 ^a		
n	14	7
Mean (SD)	-3.21 (17.89)	-2.68 (17.53)
Median	-2.61	3.12
25th, 75th Percentile	-12.50, 12.50	-12.50, 6.25
Min, Max	-36.6, 25.0	-37.5, 15.6
Difference in change from baseline (95%CI)		0.53 (-16.70, 17.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9492
Hedges'g (95% CI) ^c		0.03 (-0.88, 0.94)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	8	11
Mean (SD)	76.83 (18.51)	76.42 (13.43)
Median	82.82	81.25
25th, 75th Percentile	64.07, 89.07	62.50, 84.38
Min, Max	46.9, 95.8	56.3, 93.8
Week 26		
n	13	10
Mean (SD)	74.04 (23.01)	77.81 (13.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	79.69
25th, 75th Percentile	56.25, 90.63	68.75, 87.50
Min, Max	31.3, 100.0	56.3, 100.0
Change from baseline to Week 26 ^a		
n	8	10
Mean (SD)	1.30 (10.32)	1.88 (14.82)
Median	2.09	4.69
25th, 75th Percentile	-3.13, 7.81	-9.38, 9.38
Min, Max	-18.8, 15.6	-25.0, 25.0
Week 52		
n	14	14
Mean (SD)	69.42 (16.99)	75.90 (10.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.63	75.00
25th, 75th Percentile	56.25, 81.25	68.75, 84.38
Min, Max	43.8, 100.0	59.4, 96.9
Change from baseline to Week 52 ^a		
n	7	10
Mean (SD)	-1.64 (10.84)	1.25 (14.60)
Median	0.00	-3.13
25th, 75th Percentile	-12.50, 6.25	-9.38, 6.25
Min, Max	-15.6, 15.6	-12.5, 31.3
Difference in change from baseline (95%CI)		2.89 (-11.00, 16.78)
P-value ^b		0.6641

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.002.006_qs_sum_ovr_ped_self_phy_bhagv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.2.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.21 (-0.76, 1.17)
P-value for interaction term, treatment * [Baseline AGV]		0.9727

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	24	22
Mean (SD)	75.78 (17.85)	76.60 (14.86)
Median	78.13	80.73
25th, 75th Percentile	62.50, 91.75	62.50, 90.63
Min, Max	31.3, 100.0	50.0, 96.9
Week 26		
n	27	25
Mean (SD)	75.18 (18.80)	76.63 (14.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	81.25	78.13
25th, 75th Percentile	68.75, 90.63	65.63, 87.50
Min, Max	31.3, 96.9	43.8, 100.0
Change from baseline to Week 26 ^a		
n	22	21
Mean (SD)	1.18 (11.72)	-0.04 (11.72)
Median	4.69	3.12
25th, 75th Percentile	-3.13, 9.37	-6.25, 6.25
Min, Max	-21.9, 18.8	-25.0, 25.0
Week 52		
n	30	27
Mean (SD)	72.54 (14.70)	76.54 (14.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.44	78.13
25th, 75th Percentile	62.50, 81.25	68.75, 87.50
Min, Max	43.8, 100.0	46.4, 100.0
Change from baseline to Week 52 ^a		
n	23	21
Mean (SD)	-0.91 (16.13)	-0.89 (14.74)
Median	3.12	-3.12
25th, 75th Percentile	-12.50, 9.38	-9.38, 4.17
Min, Max	-36.6, 25.0	-37.5, 31.3
Difference in change from baseline (95%CI)		0.02 (-9.41, 9.45)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9966
Hedges'g (95% CI) ^c		0.00 (-0.59, 0.59)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported PedsQL : Physical Health Summary Score		
Baseline		
n	11	6
Mean (SD)	79.74 (17.98)	80.21 (11.64)
Median	84.38	81.25
25th, 75th Percentile	65.63, 95.83	71.88, 90.63
Min, Max	40.6, 96.9	62.5, 93.8
Week 26		
n	12	5
Mean (SD)	70.58 (21.17)	75.00 (8.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	75.00
25th, 75th Percentile	53.13, 85.94	68.75, 78.13
Min, Max	34.4, 100.0	65.6, 87.5
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	-6.46 (11.46)	-5.00 (14.92)
Median	-6.25	-6.25
25th, 75th Percentile	-15.63, -3.12	-12.50, 6.25
Min, Max	-21.9, 15.6	-25.0, 12.5
Week 52		
n	13	7
Mean (SD)	70.43 (21.07)	81.25 (15.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.75	84.38
25th, 75th Percentile	53.13, 87.50	62.50, 96.88
Min, Max	37.5, 96.9	59.4, 96.9
Change from baseline to Week 52 ^a		
n	10	4
Mean (SD)	-4.59 (17.17)	3.13 (10.52)
Median	-7.82	3.13
25th, 75th Percentile	-15.63, 1.05	-4.69, 10.94
Min, Max	-28.1, 31.3	-9.4, 15.6
Difference in change from baseline (95%CI)		7.71 (-12.62, 28.04)
P-value ^b		0.4247

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.2.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Physical Health Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.46 (-0.73, 1.62)
P-value for interaction term, treatment * [Ethnicity]		0.4596

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	20	15
Mean (SD)	73.87 (15.95)	72.11 (15.92)
Median	78.33	73.33
25th, 75th Percentile	59.17, 87.50	70.00, 80.00
Min, Max	45.0, 93.3	26.7, 93.3
Week 26		
n	19	14
Mean (SD)	73.42 (13.25)	70.06 (16.17)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.33	68.33
25th, 75th Percentile	63.33, 85.00	65.00, 83.33
Min, Max	43.3, 98.3	30.0, 89.3
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-2.20 (12.07)	-1.49 (10.07)
Median	0.00	-3.33
25th, 75th Percentile	-8.34, 6.43	-6.67, 3.33
Min, Max	-28.3, 20.0	-21.8, 16.0
Week 52		
n	23	17
Mean (SD)	71.81 (14.55)	71.79 (14.99)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.33	75.00
25th, 75th Percentile	60.00, 83.33	66.67, 83.33
Min, Max	46.7, 100.0	43.3, 88.3
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	-2.35 (18.60)	-0.80 (16.23)
Median	-0.84	-0.84
25th, 75th Percentile	-6.67, 8.34	-10.00, 6.66
Min, Max	-41.7, 25.0	-30.0, 30.0
Difference in change from baseline (95%CI)		1.55 (-11.26, 14.37)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8065
Hedges'g (95% CI) ^c		0.09 (-0.61, 0.78)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	15	13
Mean (SD)	75.21 (14.77)	72.57 (11.48)
Median	71.67	71.67
25th, 75th Percentile	65.00, 93.33	65.00, 81.67
Min, Max	48.3, 98.2	50.0, 88.3
Week 26		
n	20	16
Mean (SD)	76.95 (14.61)	73.96 (11.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	70.83
25th, 75th Percentile	66.67, 87.50	65.84, 83.33
Min, Max	50.0, 98.3	51.7, 91.7
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	3.38 (8.83)	3.33 (12.75)
Median	3.33	0.83
25th, 75th Percentile	-2.74, 8.33	-6.67, 7.50
Min, Max	-18.3, 15.0	-8.3, 33.3
Week 52		
n	20	17
Mean (SD)	70.12 (12.30)	78.33 (11.26)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.50	78.33
25th, 75th Percentile	57.50, 79.17	73.33, 86.67
Min, Max	51.7, 90.0	60.0, 96.7
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	-3.72 (14.51)	4.09 (16.40)
Median	-3.34	3.33
25th, 75th Percentile	-5.00, 3.33	-5.00, 16.66
Min, Max	-44.9, 24.0	-26.7, 36.7
Difference in change from baseline (95%CI)		7.81 (-4.75, 20.37)
P-value ^b		0.2114

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.49 (-0.30, 1.28)
P-value for interaction term, treatment * [Sex]		0.4834

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Psychosocial summary score is the mean score (sum of items scored/number of items answered in the emotional, social and school functioning domains).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.003.001_qs_sum_ovr_ped_self_psy_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Week 26		
n	5	4
Mean (SD)	67.67 (14.46)	72.08 (17.71)
Median	61.67	73.33
25th, 75th Percentile	61.67, 80.00	57.50, 86.67
Min, Max	50.0, 85.0	51.7, 90.0
Week 52		
n	8	8
Mean (SD)	62.92 (13.09)	80.83 (10.58)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	60.00	77.50
25th, 75th Percentile	52.50, 75.00	74.17, 90.00
Min, Max	46.7, 81.7	66.7, 96.7
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	23	16
Mean (SD)	72.90 (15.65)	71.56 (8.72)
Median	73.33	72.50
25th, 75th Percentile	58.33, 86.67	65.84, 75.84
Min, Max	45.0, 98.2	51.7, 88.3
Week 26		
n	23	15
Mean (SD)	74.37 (15.03)	70.83 (10.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.33	68.33
25th, 75th Percentile	63.33, 85.00	65.00, 76.67
Min, Max	43.3, 98.3	48.2, 91.7
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	-0.28 (11.23)	0.39 (10.99)
Median	0.83	-1.67
25th, 75th Percentile	-5.00, 8.33	-6.67, 6.67
Min, Max	-28.3, 20.0	-21.8, 20.0
Week 52		
n	23	15
Mean (SD)	70.61 (12.44)	70.22 (15.25)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.33	75.00
25th, 75th Percentile	60.00, 81.67	60.00, 85.00
Min, Max	46.7, 90.0	43.3, 88.3
Change from baseline to Week 52 ^a		
n	22	14
Mean (SD)	-3.69 (17.25)	-1.31 (12.80)
Median	-1.67	0.00
25th, 75th Percentile	-8.33, 4.76	-5.00, 5.00
Min, Max	-44.9, 24.0	-30.0, 20.0
Difference in change from baseline (95%CI)		2.38 (-8.53, 13.28)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6609
Hedges'g (95% CI) ^c		0.15 (-0.52, 0.82)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	12	12
Mean (SD)	77.40 (14.64)	73.33 (18.99)
Median	78.57	80.00
25th, 75th Percentile	71.67, 89.17	67.50, 87.50
Min, Max	45.0, 95.0	26.7, 93.3
Week 26		
n	11	11
Mean (SD)	80.45 (9.64)	73.94 (16.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	83.33	81.67
25th, 75th Percentile	71.67, 86.67	66.67, 83.33
Min, Max	63.3, 96.7	30.0, 86.7
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	1.95 (10.48)	1.21 (12.49)
Median	4.76	-1.66
25th, 75th Percentile	-5.00, 8.33	-6.67, 3.33
Min, Max	-15.0, 15.0	-11.7, 33.3
Week 52		
n	12	11
Mean (SD)	77.22 (13.40)	77.46 (11.25)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.50	81.67
25th, 75th Percentile	70.00, 85.84	68.75, 86.67
Min, Max	46.7, 100.0	56.7, 88.3
Change from baseline to Week 52 ^a		
n	11	11
Mean (SD)	-1.56 (16.01)	4.73 (19.77)
Median	-2.14	3.33
25th, 75th Percentile	-5.00, 8.33	-10.00, 23.33
Min, Max	-41.7, 25.0	-26.7, 36.7
Difference in change from baseline (95%CI)		6.29 (-9.71, 22.29)
P-value ^b		0.4216

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.34 (-0.51, 1.17)
P-value for interaction term, treatment * [Age at Baseline]		0.6672

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	22	16
Mean (SD)	75.31 (13.98)	68.65 (14.26)
Median	77.50	70.84
25th, 75th Percentile	66.67, 86.67	65.00, 77.50
Min, Max	45.0, 95.0	26.7, 88.3
Week 26		
n	27	19
Mean (SD)	73.95 (13.81)	69.78 (15.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.33	68.33
25th, 75th Percentile	63.33, 83.33	63.33, 83.33
Min, Max	43.3, 98.3	30.0, 91.7
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	-1.01 (11.87)	1.28 (10.86)
Median	1.67	3.33
25th, 75th Percentile	-8.34, 8.33	-5.00, 6.67
Min, Max	-28.3, 20.0	-21.8, 20.0
Week 52		
n	31	23
Mean (SD)	70.22 (12.94)	74.35 (15.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.33	76.67
25th, 75th Percentile	60.00, 81.67	61.67, 85.00
Min, Max	46.7, 90.0	43.3, 96.7
Change from baseline to Week 52 ^a		
n	21	14
Mean (SD)	-4.14 (15.90)	2.14 (16.50)
Median	-1.67	2.50
25th, 75th Percentile	-6.67, 3.33	-5.00, 8.34
Min, Max	-41.7, 18.3	-30.0, 30.0
Difference in change from baseline (95%CI)		6.28 (-5.05, 17.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2675
Hedges'g (95% CI) ^c		0.38 (-0.30, 1.06)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	13	12
Mean (SD)	72.98 (17.69)	77.22 (11.96)
Median	71.67	78.34
25th, 75th Percentile	60.00, 90.00	70.84, 87.50
Min, Max	45.0, 98.2	50.0, 93.3
Week 26		
n	12	11
Mean (SD)	78.10 (14.24)	76.21 (7.89)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	83.33	80.00
25th, 75th Percentile	68.33, 87.50	68.33, 83.33
Min, Max	50.0, 95.0	65.0, 85.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	2.80 (8.97)	0.00 (12.63)
Median	2.50	-5.00
25th, 75th Percentile	-2.98, 10.00	-8.33, 3.33
Min, Max	-15.0, 15.0	-8.3, 33.3
Week 52		
n	12	11
Mean (SD)	73.11 (14.99)	76.55 (9.26)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	74.17	76.67
25th, 75th Percentile	60.83, 82.86	68.75, 86.67
Min, Max	51.7, 100.0	60.0, 88.3
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	-0.94 (18.37)	0.34 (16.43)
Median	-2.74	0.00
25th, 75th Percentile	-5.00, 8.33	-10.00, 5.00
Min, Max	-44.9, 25.0	-26.7, 36.7
Difference in change from baseline (95%CI)		1.28 (-13.88, 16.45)
P-value ^b		0.8620

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.07 (-0.75, 0.89)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5822

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	4	8
Mean (SD)	68.75 (12.50)	75.83 (9.26)
Median	70.84	74.17
25th, 75th Percentile	60.84, 76.67	68.34, 84.17
Min, Max	51.7, 81.7	65.0, 88.3
Week 26		
n	6	7
Mean (SD)	63.06 (15.65)	75.71 (11.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	62.50	68.33
25th, 75th Percentile	50.00, 73.33	68.33, 86.67
Min, Max	43.3, 86.7	63.3, 91.7
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-2.09 (14.43)	4.72 (9.97)
Median	-2.51	3.33
25th, 75th Percentile	-13.34, 9.17	-1.66, 11.66
Min, Max	-18.3, 15.0	-8.3, 20.0
Week 52		
n	6	9
Mean (SD)	67.78 (9.76)	80.19 (10.29)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.33	81.67
25th, 75th Percentile	65.00, 71.67	75.00, 86.67
Min, Max	51.7, 81.7	60.0, 95.0
Change from baseline to Week 52 ^a		
n	4	5
Mean (SD)	3.75 (8.86)	5.00 (12.47)
Median	0.84	0.00
25th, 75th Percentile	-1.67, 9.17	-5.00, 16.66
Min, Max	-3.3, 16.7	-6.7, 20.0
Difference in change from baseline (95%CI)		1.25 (-16.30, 18.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8708
Hedges'g (95% CI) ^c		0.10 (-1.22, 1.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	14	8
Mean (SD)	74.93 (14.79)	65.63 (16.81)
Median	77.62	70.00
25th, 75th Percentile	60.00, 86.67	64.17, 74.17
Min, Max	45.0, 95.0	26.7, 81.7
Week 26		
n	13	8
Mean (SD)	79.87 (11.13)	65.31 (18.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	65.84
25th, 75th Percentile	73.33, 85.00	56.61, 79.17
Min, Max	61.7, 98.3	30.0, 89.3
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	1.90 (9.74)	-0.31 (10.92)
Median	3.33	0.83
25th, 75th Percentile	-3.34, 8.33	-4.17, 5.00
Min, Max	-15.0, 15.0	-21.8, 16.0
Week 52		
n	16	9
Mean (SD)	70.73 (14.48)	64.44 (14.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	74.17	66.67
25th, 75th Percentile	55.83, 81.67	56.67, 73.33
Min, Max	46.7, 90.0	43.3, 88.3
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-2.36 (17.35)	-1.46 (18.80)
Median	-2.14	1.67
25th, 75th Percentile	-5.00, 4.76	-12.50, 5.00
Min, Max	-41.7, 25.0	-30.0, 30.0
Difference in change from baseline (95%CI)		0.90 (-15.93, 17.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9118
Hedges'g (95% CI) ^c		0.05 (-0.83, 0.93)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	11	8
Mean (SD)	73.17 (15.75)	70.83 (13.57)
Median	71.67	73.33
25th, 75th Percentile	61.67, 90.00	60.84, 80.00
Min, Max	48.3, 98.2	50.0, 88.3
Week 26		
n	14	11
Mean (SD)	74.80 (12.01)	73.48 (11.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	68.33
25th, 75th Percentile	70.00, 83.33	66.67, 83.33
Min, Max	50.0, 95.0	51.7, 90.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	0.67 (8.61)	2.08 (15.06)
Median	1.67	-5.00
25th, 75th Percentile	-5.00, 8.33	-6.67, 9.17
Min, Max	-16.7, 10.0	-11.7, 33.3
Week 52		
n	16	12
Mean (SD)	69.21 (13.33)	77.67 (11.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	71.67	79.17
25th, 75th Percentile	57.50, 79.17	71.88, 85.00
Min, Max	46.7, 90.0	50.0, 96.7
Change from baseline to Week 52 ^a		
n	11	8
Mean (SD)	-5.38 (19.49)	3.60 (15.05)
Median	-5.00	1.67
25th, 75th Percentile	-8.33, 3.34	-6.67, 6.67
Min, Max	-44.9, 24.0	-11.3, 36.7
Difference in change from baseline (95%CI)		8.97 (-8.47, 26.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2930
Hedges'g (95% CI) ^c		0.48 (-0.45, 1.40)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	6	4
Mean (SD)	79.44 (19.02)	81.67 (12.09)
Median	85.00	84.17
25th, 75th Percentile	73.33, 93.33	73.34, 90.00
Min, Max	45.0, 95.0	65.0, 93.3
Week 26		
n	6	4
Mean (SD)	78.33 (17.45)	75.83 (8.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	76.67
25th, 75th Percentile	65.00, 93.33	69.17, 82.50
Min, Max	53.3, 98.3	65.0, 85.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	-1.11 (16.08)	-5.84 (3.97)
Median	-0.84	-7.50
25th, 75th Percentile	-5.00, 8.33	-8.34, -3.34
Min, Max	-28.3, 20.0	-8.3, 0.0
Week 52		
n	5	4
Mean (SD)	81.67 (12.19)	79.58 (13.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.33	85.00
25th, 75th Percentile	75.00, 86.67	71.67, 87.50
Min, Max	68.3, 100.0	60.0, 88.3
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	-4.67 (15.30)	-2.09 (21.32)
Median	1.67	-2.50
25th, 75th Percentile	-16.67, 8.33	-18.34, 14.17
Min, Max	-25.0, 8.3	-26.7, 23.3
Difference in change from baseline (95%CI)		2.58 (-26.17, 31.33)
P-value ^b		0.8379

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.3.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.13 (-1.19, 1.44)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.8899

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	13	9
Mean (SD)	71.15 (13.92)	67.59 (17.02)
Median	71.67	73.33
25th, 75th Percentile	61.67, 78.33	65.00, 80.00
Min, Max	48.3, 93.3	26.7, 81.7
Week 26		
n	14	10
Mean (SD)	68.97 (12.73)	67.67 (15.74)

Max, maximum; Min, minimum; SD, standard deviation.

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^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	71.67	68.33
25th, 75th Percentile	61.67, 78.33	65.00, 76.67
Min, Max	43.3, 86.7	30.0, 90.0
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-1.62 (11.58)	-0.63 (6.10)
Median	1.66	1.67
25th, 75th Percentile	-8.33, 3.33	-5.00, 3.33
Min, Max	-28.3, 15.0	-11.7, 6.7
Week 52		
n	14	11
Mean (SD)	66.60 (12.83)	76.52 (12.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.33	75.00
25th, 75th Percentile	58.33, 73.33	61.67, 88.33
Min, Max	46.7, 86.7	56.7, 96.7
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-3.69 (17.19)	8.54 (12.10)
Median	-3.33	5.83
25th, 75th Percentile	-14.17, 5.84	0.00, 15.84
Min, Max	-31.7, 24.0	-5.0, 30.0
Difference in change from baseline (95%CI)		12.23 (-2.55, 27.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0991
Hedges'g (95% CI) ^c		0.76 (-0.18, 1.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	14	8
Mean (SD)	74.56 (15.97)	76.04 (8.90)
Median	74.17	72.50
25th, 75th Percentile	58.33, 88.33	70.00, 85.00
Min, Max	45.0, 95.0	65.0, 88.3
Week 26		
n	12	10
Mean (SD)	79.03 (11.09)	73.75 (15.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.34	77.50
25th, 75th Percentile	72.50, 87.50	63.33, 86.67
Min, Max	61.7, 96.7	48.2, 91.7
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	3.14 (8.33)	-0.73 (13.61)
Median	6.19	-4.16
25th, 75th Percentile	-1.67, 8.33	-7.51, 9.65
Min, Max	-15.0, 15.0	-21.8, 20.0
Week 52		
n	15	9
Mean (SD)	72.56 (14.43)	73.33 (16.94)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	80.00
25th, 75th Percentile	60.00, 80.00	75.00, 85.00
Min, Max	46.7, 100.0	43.3, 86.7
Change from baseline to Week 52 ^a		
n	14	7
Mean (SD)	-0.63 (16.44)	-5.71 (17.61)
Median	-1.91	-6.66
25th, 75th Percentile	-5.00, 8.33	-25.00, 5.00
Min, Max	-41.7, 25.0	-30.0, 20.0
Difference in change from baseline (95%CI)		-5.08 (-21.38, 11.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.5217
Hedges'g (95% CI) ^c		-0.29 (-1.20, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	8	11
Mean (SD)	79.60 (16.52)	73.49 (13.89)
Median	80.12	73.33
25th, 75th Percentile	75.00, 91.67	66.67, 86.67
Min, Max	45.0, 98.2	50.0, 93.3
Week 26		
n	13	10
Mean (SD)	78.46 (15.86)	75.00 (8.57)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	74.17
25th, 75th Percentile	70.00, 88.33	66.67, 83.33
Min, Max	50.0, 98.3	65.0, 85.0
Change from baseline to Week 26 ^a		
n	8	10
Mean (SD)	-0.02 (13.25)	3.00 (13.40)
Median	1.61	-2.50
25th, 75th Percentile	-10.84, 8.33	-6.67, 11.66
Min, Max	-18.3, 20.0	-8.3, 33.3
Week 52		
n	14	14
Mean (SD)	73.81 (12.68)	75.03 (12.27)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.84	75.00
25th, 75th Percentile	66.67, 83.33	68.33, 86.67
Min, Max	51.7, 90.0	50.0, 95.0
Change from baseline to Week 52 ^a		
n	7	10
Mean (SD)	-6.44 (17.84)	0.54 (17.02)
Median	0.00	-0.84
25th, 75th Percentile	-8.33, 4.76	-10.00, 3.33
Min, Max	-44.9, 8.3	-26.7, 36.7
Difference in change from baseline (95%CI)		6.98 (-11.24, 25.21)
P-value ^b		0.4269

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.38 (-0.60, 1.35)
P-value for interaction term, treatment * [Baseline AGV]		0.2659

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	24	22
Mean (SD)	73.01 (14.13)	70.76 (14.88)
Median	72.50	72.50
25th, 75th Percentile	65.84, 84.41	65.00, 80.00
Min, Max	45.0, 95.0	26.7, 93.3
Week 26		
n	27	25
Mean (SD)	75.76 (12.49)	71.37 (14.06)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	68.33
25th, 75th Percentile	70.00, 83.33	65.00, 83.33
Min, Max	50.0, 98.3	30.0, 90.0
Change from baseline to Week 26 ^a		
n	22	21
Mean (SD)	1.44 (10.57)	0.91 (11.53)
Median	2.50	-1.66
25th, 75th Percentile	-3.34, 8.33	-5.00, 3.33
Min, Max	-18.3, 20.0	-21.8, 33.3
Week 52		
n	30	27
Mean (SD)	71.91 (12.71)	73.95 (14.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Psychosocial summary score is the mean score (sum of items scored/number of items answered in the emotional, social and school functioning domains).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	74.17	76.67
25th, 75th Percentile	65.00, 81.67	61.67, 85.00
Min, Max	46.7, 90.0	43.3, 96.7
Change from baseline to Week 52 ^a		
n	23	21
Mean (SD)	-1.80 (15.55)	1.67 (17.42)
Median	-1.67	0.00
25th, 75th Percentile	-5.00, 3.34	-6.66, 8.34
Min, Max	-41.7, 25.0	-30.0, 36.7
Difference in change from baseline (95%CI)		3.47 (-6.56, 13.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Psychosocial summary score is the mean score (sum of items scored/number of items answered in the emotional, social and school functioning domains).

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.4893
Hedges'g (95% CI) ^c		0.21 (-0.39, 0.80)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported PedsQL : Psychosocial Health Summary Score		
Baseline		
n	11	6
Mean (SD)	77.59 (17.76)	78.06 (6.78)
Median	78.57	78.34
25th, 75th Percentile	58.33, 93.33	71.67, 81.67
Min, Max	51.7, 98.2	70.0, 88.3
Week 26		
n	12	5
Mean (SD)	74.03 (17.20)	76.00 (11.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	78.33	73.33
25th, 75th Percentile	62.50, 87.50	68.33, 83.33
Min, Max	43.3, 95.0	63.3, 91.7
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	-1.85 (11.78)	0.00 (12.19)
Median	-2.44	-6.67
25th, 75th Percentile	-5.00, 6.43	-8.34, 3.33
Min, Max	-28.3, 15.0	-8.3, 20.0
Week 52		
n	13	7
Mean (SD)	68.97 (15.28)	79.35 (10.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.33	76.67
25th, 75th Percentile	53.33, 78.33	68.75, 86.67
Min, Max	51.7, 100.0	66.7, 95.0
Change from baseline to Week 52 ^a		
n	10	4
Mean (SD)	-5.68 (19.49)	-0.31 (7.66)
Median	-5.00	2.50
25th, 75th Percentile	-16.67, 8.33	-5.63, 5.00
Min, Max	-44.9, 18.3	-11.3, 5.0
Difference in change from baseline (95%CI)		5.37 (-16.95, 27.68)
P-value ^b		0.6098

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.3.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Psychosocial Summary Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.29 (-0.88, 1.45)
P-value for interaction term, treatment * [Ethnicity]		0.8644

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	20	15
Mean (SD)	76.50 (19.27)	74.33 (17.92)
Median	82.50	75.00
25th, 75th Percentile	57.50, 92.50	70.00, 85.00
Min, Max	40.0, 100.0	25.0, 100.0
Week 26		
n	19	14
Mean (SD)	75.53 (19.00)	72.68 (19.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	75.00
25th, 75th Percentile	55.00, 90.00	62.50, 85.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-2.06 (11.46)	-0.89 (17.20)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 5.00	-12.50, 15.00
Min, Max	-20.0, 15.0	-30.0, 25.0
Week 52		
n	23	16
Mean (SD)	75.65 (17.60)	77.50 (20.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	80.00
25th, 75th Percentile	65.00, 85.00	62.50, 95.00
Min, Max	35.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	18	13
Mean (SD)	0.56 (17.23)	2.31 (15.63)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 15.00
Min, Max	-35.0, 35.0	-25.0, 25.0
Difference in change from baseline (95%CI)		1.75 (-10.59, 14.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.7737
Hedges'g (95% CI) ^c		0.10 (-0.61, 0.82)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	15	13
Mean (SD)	76.00 (17.75)	76.15 (15.30)
Median	70.00	70.00
25th, 75th Percentile	60.00, 95.00	65.00, 90.00
Min, Max	50.0, 100.0	55.0, 100.0
Week 26		
n	20	16
Mean (SD)	78.50 (21.53)	74.69 (18.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	75.00
25th, 75th Percentile	57.50, 97.50	65.00, 90.00
Min, Max	35.0, 100.0	25.0, 95.0
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	5.00 (16.15)	4.17 (18.69)
Median	0.00	0.00
25th, 75th Percentile	0.00, 20.00	-10.00, 17.50
Min, Max	-25.0, 30.0	-25.0, 40.0
Week 52		
n	20	17
Mean (SD)	73.19 (17.71)	78.14 (17.16)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	62.50, 85.00	65.00, 90.00
Min, Max	35.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	-0.42 (22.54)	1.36 (20.63)
Median	0.00	-5.00
25th, 75th Percentile	-15.00, 15.00	-10.00, 20.00
Min, Max	-50.0, 43.8	-35.0, 30.0
Difference in change from baseline (95%CI)		1.78 (-16.05, 19.61)
P-value ^b		0.8385

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.08 (-0.70, 0.86)
P-value for interaction term, treatment * [Sex]		0.9978

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported PedsQL : Emotional Functioning Score		
Week 26		
n	5	4
Mean (SD)	62.00 (17.54)	62.50 (27.23)
Median	55.00	70.00
25th, 75th Percentile	55.00, 80.00	42.50, 82.50
Min, Max	40.0, 80.0	25.0, 85.0
Week 52		
n	8	8
Mean (SD)	56.25 (18.47)	78.54 (16.27)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	57.50	77.50
25th, 75th Percentile	37.50, 72.50	66.67, 92.50
Min, Max	35.0, 80.0	55.0, 100.0
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	23	16
Mean (SD)	76.30 (19.44)	72.19 (12.64)
Median	85.00	70.00
25th, 75th Percentile	55.00, 95.00	65.00, 75.00
Min, Max	40.0, 100.0	55.0, 95.0
Week 26		
n	23	15
Mean (SD)	75.87 (21.30)	75.50 (15.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	75.00
25th, 75th Percentile	55.00, 95.00	65.00, 90.00
Min, Max	35.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	-1.82 (12.11)	4.83 (17.79)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 5.00	-10.00, 20.00
Min, Max	-25.0, 20.0	-30.0, 40.0
Week 52		
n	23	15
Mean (SD)	74.51 (14.22)	76.00 (17.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	70.00
25th, 75th Percentile	65.00, 85.00	60.00, 95.00
Min, Max	50.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	22	14
Mean (SD)	-3.69 (20.52)	2.50 (15.54)
Median	-2.50	0.00
25th, 75th Percentile	-10.00, 5.00	-5.00, 15.00
Min, Max	-50.0, 43.8	-25.0, 30.0
Difference in change from baseline (95%CI)		6.19 (-6.85, 19.23)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.3413
Hedges'g (95% CI) ^c		0.32 (-0.35, 0.99)

Max, maximum; Min, minimum; SD, standard deviation.

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^b Two-sided p-value.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	12	12
Mean (SD)	76.25 (16.94)	79.17 (20.43)
Median	80.00	85.00
25th, 75th Percentile	60.00, 90.00	72.50, 92.50
Min, Max	50.0, 100.0	25.0, 100.0
Week 26		
n	11	11
Mean (SD)	86.36 (14.33)	75.45 (19.16)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	90.00	75.00
25th, 75th Percentile	85.00, 100.00	70.00, 90.00
Min, Max	55.0, 100.0	30.0, 95.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	8.00 (16.36)	-3.18 (17.36)
Median	5.00	-5.00
25th, 75th Percentile	-5.00, 25.00	-15.00, 15.00
Min, Max	-15.0, 30.0	-25.0, 25.0
Week 52		
n	12	10
Mean (SD)	86.67 (11.93)	80.00 (21.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	90.00
25th, 75th Percentile	80.00, 97.50	70.00, 95.00
Min, Max	65.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	11	10
Mean (SD)	7.73 (15.39)	1.00 (21.19)
Median	5.00	2.50
25th, 75th Percentile	0.00, 15.00	-10.00, 20.00
Min, Max	-15.0, 35.0	-35.0, 25.0
Difference in change from baseline (95%CI)		-6.73 (-23.52, 10.07)
P-value ^b		0.4122

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.2
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Emotional Functioning Score for BMN111-301
 Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.35 (-1.21, 0.52)
P-value for interaction term, treatment * [Age at Baseline]		0.2166

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	22	16
Mean (SD)	77.73 (17.71)	71.25 (17.46)
Median	82.50	72.50
25th, 75th Percentile	65.00, 90.00	65.00, 80.00
Min, Max	40.0, 100.0	25.0, 100.0
Week 26		
n	27	19
Mean (SD)	75.19 (19.88)	70.66 (20.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	70.00
25th, 75th Percentile	55.00, 95.00	60.00, 85.00
Min, Max	40.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	-1.25 (13.85)	2.50 (19.11)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 7.50	-10.00, 20.00
Min, Max	-25.0, 30.0	-30.0, 40.0
Week 52		
n	31	23
Mean (SD)	73.06 (18.01)	77.54 (20.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	80.00
25th, 75th Percentile	65.00, 85.00	58.33, 95.00
Min, Max	35.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	21	14
Mean (SD)	-1.43 (15.42)	3.93 (16.66)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 15.00	-5.00, 15.00
Min, Max	-35.0, 20.0	-25.0, 30.0
Difference in change from baseline (95%CI)		5.36 (-5.82, 16.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.3366
Hedges'g (95% CI) ^c		0.33 (-0.35, 1.01)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	13	12
Mean (SD)	73.85 (19.91)	80.42 (14.05)
Median	70.00	80.00
25th, 75th Percentile	55.00, 95.00	70.00, 92.50
Min, Max	50.0, 100.0	55.0, 100.0
Week 26		
n	12	11
Mean (SD)	81.25 (20.90)	79.09 (12.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	90.00	75.00
25th, 75th Percentile	70.00, 92.50	70.00, 90.00
Min, Max	35.0, 100.0	60.0, 95.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	5.42 (14.05)	0.00 (16.43)
Median	0.00	0.00
25th, 75th Percentile	-2.50, 17.50	-15.00, 15.00
Min, Max	-15.0, 30.0	-25.0, 25.0
Week 52		
n	12	10
Mean (SD)	78.23 (16.17)	78.50 (14.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	77.50
25th, 75th Percentile	70.00, 89.38	70.00, 90.00
Min, Max	50.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	12	10
Mean (SD)	2.81 (25.71)	-1.00 (19.55)
Median	0.00	0.00
25th, 75th Percentile	-7.50, 20.00	-10.00, 15.00
Min, Max	-50.0, 43.8	-35.0, 25.0
Difference in change from baseline (95%CI)		-3.81 (-24.48, 16.86)
P-value ^b		0.7045

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.16 (-1.00, 0.68)
P-value for interaction term, treatment *[Baseline Tanner Stage]		0.3834

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	4	8
Mean (SD)	73.75 (17.97)	77.50 (14.88)
Median	77.50	72.50
25th, 75th Percentile	60.00, 87.50	70.00, 90.00
Min, Max	50.0, 90.0	55.0, 100.0
Week 26		
n	6	7
Mean (SD)	62.50 (20.19)	78.57 (14.92)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	57.50	85.00
25th, 75th Percentile	55.00, 65.00	60.00, 90.00
Min, Max	40.0, 100.0	60.0, 95.0
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-7.50 (16.58)	8.33 (20.66)
Median	-10.00	7.50
25th, 75th Percentile	-17.50, 2.50	-10.00, 20.00
Min, Max	-25.0, 15.0	-15.0, 40.0
Week 52		
n	6	9
Mean (SD)	66.67 (11.69)	83.15 (16.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	67.50	90.00
25th, 75th Percentile	60.00, 70.00	70.00, 95.00
Min, Max	50.0, 85.0	58.3, 100.0
Change from baseline to Week 52 ^a		
n	4	5
Mean (SD)	-2.50 (15.55)	7.00 (16.81)
Median	-7.50	-5.00
25th, 75th Percentile	-12.50, 7.50	-5.00, 20.00
Min, Max	-15.0, 20.0	-5.0, 30.0
Difference in change from baseline (95%CI)		9.50 (-16.32, 35.32)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.4132
Hedges'g (95% CI) ^c		0.52 (-0.84, 1.84)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	14	8
Mean (SD)	76.79 (15.89)	69.38 (22.59)
Median	80.00	72.50
25th, 75th Percentile	65.00, 85.00	60.00, 85.00
Min, Max	50.0, 100.0	25.0, 95.0
Week 26		
n	13	8
Mean (SD)	82.69 (18.10)	70.31 (20.89)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	90.00	67.50
25th, 75th Percentile	85.00, 90.00	63.75, 85.00
Min, Max	40.0, 100.0	30.0, 100.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	5.45 (15.40)	0.94 (16.25)
Median	5.00	2.50
25th, 75th Percentile	-10.00, 20.00	-11.25, 12.50
Min, Max	-15.0, 30.0	-25.0, 25.0
Week 52		
n	16	9
Mean (SD)	76.56 (19.30)	66.11 (18.33)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	82.50	70.00
25th, 75th Percentile	67.50, 85.00	55.00, 75.00
Min, Max	35.0, 100.0	35.0, 95.0
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	4.23 (18.24)	-4.38 (17.20)
Median	0.00	-2.50
25th, 75th Percentile	-5.00, 15.00	-22.50, 12.50
Min, Max	-35.0, 35.0	-25.0, 15.0
Difference in change from baseline (95%CI)		-8.61 (-25.41, 8.20)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2972
Hedges'g (95% CI) ^c		-0.46 (-1.35, 0.44)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	11	8
Mean (SD)	72.73 (21.02)	73.75 (13.82)
Median	60.00	72.50
25th, 75th Percentile	55.00, 95.00	65.00, 80.00
Min, Max	50.0, 100.0	55.0, 100.0
Week 26		
n	14	11
Mean (SD)	74.64 (21.16)	72.27 (21.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	82.50	75.00
25th, 75th Percentile	55.00, 85.00	60.00, 90.00
Min, Max	35.0, 100.0	25.0, 95.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	0.00 (12.65)	1.88 (20.69)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 5.00	-15.00, 20.00
Min, Max	-20.0, 25.0	-30.0, 25.0
Week 52		
n	16	11
Mean (SD)	71.48 (17.66)	81.36 (15.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.50	90.00
25th, 75th Percentile	57.50, 85.00	70.00, 90.00
Min, Max	40.0, 100.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	11	7
Mean (SD)	-1.48 (25.28)	7.86 (14.10)
Median	0.00	5.00
25th, 75th Percentile	-20.00, 15.00	-5.00, 25.00
Min, Max	-50.0, 43.8	-10.0, 25.0
Difference in change from baseline (95%CI)		9.33 (-12.98, 31.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.3883
Hedges'g (95% CI) ^c		0.41 (-0.56, 1.36)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	6	4
Mean (SD)	83.33 (21.60)	85.00 (7.07)
Median	92.50	87.50
25th, 75th Percentile	85.00, 95.00	80.00, 90.00
Min, Max	40.0, 95.0	75.0, 90.0
Week 26		
n	6	4
Mean (SD)	85.00 (17.03)	76.25 (9.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	92.50	72.50
25th, 75th Percentile	75.00, 95.00	70.00, 82.50
Min, Max	55.0, 100.0	70.0, 90.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	1.67 (12.91)	-8.75 (7.50)
Median	5.00	-10.00
25th, 75th Percentile	-5.00, 10.00	-15.00, -2.50
Min, Max	-20.0, 15.0	-15.0, 0.0
Week 52		
n	5	4
Mean (SD)	87.00 (11.51)	82.50 (23.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	90.00
25th, 75th Percentile	85.00, 95.00	65.00, 100.00
Min, Max	70.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	-5.00 (12.75)	-2.50 (25.98)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 5.00	-22.50, 17.50
Min, Max	-25.0, 5.0	-35.0, 25.0
Difference in change from baseline (95%CI)		2.50 (-28.51, 33.51)
P-value ^b		0.8542

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.11 (-1.21, 1.43)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.4894

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	13	9
Mean (SD)	73.08 (19.85)	70.56 (19.91)
Median	70.00	70.00
25th, 75th Percentile	55.00, 90.00	65.00, 85.00
Min, Max	50.0, 100.0	25.0, 95.0
Week 26		
n	14	10
Mean (SD)	70.00 (21.66)	70.00 (17.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	67.50	70.00
25th, 75th Percentile	55.00, 90.00	60.00, 85.00
Min, Max	35.0, 100.0	30.0, 90.0
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-1.92 (13.31)	0.63 (14.25)
Median	0.00	2.50
25th, 75th Percentile	-10.00, 0.00	-7.50, 10.00
Min, Max	-20.0, 30.0	-25.0, 20.0
Week 52		
n	14	11
Mean (SD)	70.98 (16.87)	78.48 (21.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	85.00
25th, 75th Percentile	60.00, 85.00	60.00, 100.00
Min, Max	35.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-0.10 (22.84)	10.00 (12.25)
Median	-2.50	12.50
25th, 75th Percentile	-12.50, 17.50	-2.50, 20.00
Min, Max	-35.0, 43.8	-5.0, 25.0
Difference in change from baseline (95%CI)		10.10 (-8.52, 28.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2693
Hedges'g (95% CI) ^c		0.50 (-0.42, 1.40)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	14	8
Mean (SD)	74.29 (15.92)	80.00 (15.58)
Median	75.00	75.00
25th, 75th Percentile	60.00, 85.00	72.50, 95.00
Min, Max	50.0, 95.0	55.0, 100.0
Week 26		
n	12	10
Mean (SD)	79.58 (21.79)	75.25 (24.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	90.00	85.00
25th, 75th Percentile	65.00, 95.00	62.50, 95.00
Min, Max	40.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	6.36 (15.83)	0.94 (22.04)
Median	5.00	-2.50
25th, 75th Percentile	-5.00, 20.00	-11.25, 12.50
Min, Max	-20.0, 30.0	-30.0, 40.0
Week 52		
n	15	9
Mean (SD)	77.33 (17.82)	78.33 (20.77)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	90.00
25th, 75th Percentile	70.00, 85.00	55.00, 95.00
Min, Max	35.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	14	7
Mean (SD)	6.07 (14.96)	-3.57 (18.64)
Median	5.00	-5.00
25th, 75th Percentile	0.00, 15.00	-20.00, 10.00
Min, Max	-25.0, 35.0	-25.0, 30.0
Difference in change from baseline (95%CI)		-9.64 (-25.35, 6.07)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2143
Hedges'g (95% CI) ^c		-0.57 (-1.49, 0.36)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	8	11
Mean (SD)	85.00 (19.46)	75.45 (14.40)
Median	90.00	75.00
25th, 75th Percentile	82.50, 97.50	65.00, 90.00
Min, Max	40.0, 100.0	55.0, 95.0
Week 26		
n	13	10
Mean (SD)	82.31 (15.63)	76.00 (11.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	75.00
25th, 75th Percentile	80.00, 95.00	70.00, 85.00
Min, Max	55.0, 100.0	60.0, 95.0
Change from baseline to Week 26 ^a		
n	8	10
Mean (SD)	-0.63 (12.37)	2.50 (18.30)
Median	0.00	7.50
25th, 75th Percentile	-5.00, 7.50	-15.00, 20.00
Min, Max	-25.0, 15.0	-25.0, 25.0
Week 52		
n	14	13
Mean (SD)	75.00 (18.40)	76.92 (14.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.50	75.00
25th, 75th Percentile	65.00, 90.00	70.00, 90.00
Min, Max	40.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	7	9
Mean (SD)	-11.43 (18.64)	-1.11 (20.12)
Median	-5.00	0.00
25th, 75th Percentile	-20.00, 0.00	-10.00, 15.00
Min, Max	-50.0, 5.0	-35.0, 25.0
Difference in change from baseline (95%CI)		10.32 (-10.76, 31.40)
P-value ^b		0.3116

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.50 (-0.51, 1.50)
P-value for interaction term, treatment * [Baseline AGV]		0.1780

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	24	22
Mean (SD)	75.21 (18.27)	74.55 (17.31)
Median	82.50	72.50
25th, 75th Percentile	57.50, 90.00	65.00, 85.00
Min, Max	40.0, 100.0	25.0, 100.0
Week 26		
n	27	25
Mean (SD)	77.59 (19.43)	73.10 (17.67)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	75.00
25th, 75th Percentile	60.00, 95.00	65.00, 85.00
Min, Max	35.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	22	21
Mean (SD)	1.14 (14.79)	1.07 (15.42)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 15.00
Min, Max	-25.0, 30.0	-25.0, 25.0
Week 52		
n	30	27
Mean (SD)	76.63 (15.73)	76.23 (18.85)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	80.00
25th, 75th Percentile	65.00, 85.00	58.33, 90.00
Min, Max	35.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	23	21
Mean (SD)	1.47 (18.85)	2.14 (18.75)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 15.00	-10.00, 15.00
Min, Max	-35.0, 43.8	-35.0, 30.0
Difference in change from baseline (95%CI)		0.68 (-10.78, 12.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9058
Hedges'g (95% CI) ^c		0.04 (-0.56, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported PedsQL : Emotional Functioning Score		
Baseline		
n	11	6
Mean (SD)	78.64 (19.25)	77.50 (14.05)
Median	80.00	75.00
25th, 75th Percentile	60.00, 95.00	75.00, 90.00
Min, Max	50.0, 100.0	55.0, 95.0
Week 26		
n	12	5
Mean (SD)	75.83 (22.45)	77.00 (23.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	90.00
25th, 75th Percentile	55.00, 92.50	60.00, 95.00
Min, Max	40.0, 100.0	45.0, 95.0
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	1.50 (13.13)	3.00 (27.75)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 5.00	-15.00, 20.00
Min, Max	-20.0, 25.0	-30.0, 40.0
Week 52		
n	13	6
Mean (SD)	69.62 (20.86)	85.00 (14.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.004.007_qs_sum_ovr_ped_self_emo_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	85.00
25th, 75th Percentile	50.00, 85.00	70.00, 100.00
Min, Max	35.0, 100.0	70.0, 100.0
Change from baseline to Week 52 ^a		
n	10	3
Mean (SD)	-3.00 (21.63)	0.00 (8.66)
Median	0.00	-5.00
25th, 75th Percentile	-10.00, 15.00	-5.00, 10.00
Min, Max	-50.0, 20.0	-5.0, 10.0
Difference in change from baseline (95%CI)		3.00 (-25.85, 31.85)
P-value ^b		0.8231

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.4.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Emotional Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.14 (-1.15, 1.43)
P-value for interaction term, treatment * [Ethnicity]		0.8667

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Emotional functioning score is the mean score (sum of items scored/number of items answered in emotional functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	20	15
Mean (SD)	70.25 (19.77)	76.33 (18.17)
Median	75.00	80.00
25th, 75th Percentile	57.50, 87.50	60.00, 85.00
Min, Max	35.0, 100.0	40.0, 100.0
Week 26		
n	19	14
Mean (SD)	72.11 (18.58)	70.00 (19.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	75.00
25th, 75th Percentile	55.00, 90.00	55.00, 85.00
Min, Max	40.0, 95.0	30.0, 95.0
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-2.06 (18.21)	-5.71 (12.99)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 10.00	-15.00, 0.00
Min, Max	-40.0, 20.0	-30.0, 15.0
Week 52		
n	23	17
Mean (SD)	66.30 (21.49)	69.41 (22.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	50.00, 85.00	55.00, 80.00
Min, Max	15.0, 100.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	-5.83 (26.25)	-6.07 (25.66)
Median	-5.00	-5.00
25th, 75th Percentile	-15.00, 10.00	-20.00, 10.00
Min, Max	-65.0, 40.0	-70.0, 40.0
Difference in change from baseline (95%CI)		-0.24 (-19.16, 18.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9797
Hedges'g (95% CI) ^c		-0.01 (-0.71, 0.69)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	15	13
Mean (SD)	72.33 (19.81)	70.00 (21.60)
Median	70.00	70.00
25th, 75th Percentile	55.00, 90.00	60.00, 80.00
Min, Max	40.0, 100.0	15.0, 100.0
Week 26		
n	20	16
Mean (SD)	73.75 (19.25)	74.06 (12.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.50	72.50
25th, 75th Percentile	52.50, 90.00	65.00, 85.00
Min, Max	45.0, 100.0	50.0, 90.0
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	2.67 (9.23)	4.17 (20.43)
Median	0.00	2.50
25th, 75th Percentile	0.00, 10.00	-10.00, 12.50
Min, Max	-20.0, 15.0	-20.0, 55.0
Week 52		
n	20	17
Mean (SD)	66.00 (16.03)	76.18 (14.09)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.00	80.00
25th, 75th Percentile	60.00, 80.00	70.00, 85.00
Min, Max	20.0, 90.0	45.0, 95.0
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	-5.33 (20.57)	7.27 (24.84)
Median	-5.00	5.00
25th, 75th Percentile	-15.00, 5.00	-10.00, 15.00
Min, Max	-50.0, 40.0	-25.0, 70.0
Difference in change from baseline (95%CI)		12.61 (-5.78, 31.00)
P-value ^b		0.1700

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.54 (-0.25, 1.33)
P-value for interaction term, treatment * [Sex]		0.3297

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported PedsQL : Social Functioning Score		
Week 26		
n	5	4
Mean (SD)	74.00 (17.82)	80.00 (8.16)
Median	80.00	80.00
25th, 75th Percentile	70.00, 85.00	75.00, 85.00
Min, Max	45.0, 90.0	70.0, 90.0
Week 52		
n	8	8
Mean (SD)	65.63 (17.00)	74.38 (16.57)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	50.00, 80.00	62.50, 85.00
Min, Max	40.0, 85.0	45.0, 95.0
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.005.002_qs_sum_ovr_ped_self_soc_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	23	16
Mean (SD)	66.52 (20.02)	70.94 (15.08)
Median	65.00	70.00
25th, 75th Percentile	50.00, 80.00	60.00, 80.00
Min, Max	35.0, 100.0	45.0, 95.0
Week 26		
n	23	15
Mean (SD)	67.83 (19.70)	66.00 (16.06)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	60.00	65.00
25th, 75th Percentile	50.00, 90.00	55.00, 75.00
Min, Max	40.0, 100.0	30.0, 90.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	-1.59 (17.07)	-3.33 (15.31)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-20.00, 10.00
Min, Max	-40.0, 20.0	-30.0, 20.0
Week 52		
n	23	15
Mean (SD)	62.17 (20.88)	63.33 (20.15)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.00	65.00
25th, 75th Percentile	45.00, 80.00	50.00, 80.00
Min, Max	15.0, 95.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	22	14
Mean (SD)	-5.45 (26.72)	-7.14 (24.16)
Median	-7.50	-2.50
25th, 75th Percentile	-15.00, 10.00	-20.00, 10.00
Min, Max	-65.0, 40.0	-70.0, 25.0
Difference in change from baseline (95%CI)		-1.69 (-19.59, 16.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8492
Hedges'g (95% CI) ^c		-0.06 (-0.73, 0.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	12	12
Mean (SD)	80.00 (15.67)	76.67 (24.98)
Median	85.00	85.00
25th, 75th Percentile	70.00, 90.00	75.00, 90.00
Min, Max	45.0, 100.0	15.0, 100.0
Week 26		
n	11	11
Mean (SD)	83.18 (13.09)	77.73 (14.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	90.00	75.00
25th, 75th Percentile	75.00, 90.00	65.00, 90.00
Min, Max	55.0, 100.0	55.0, 95.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	4.00 (6.15)	1.82 (19.91)
Median	2.50	-5.00
25th, 75th Percentile	0.00, 10.00	-10.00, 10.00
Min, Max	-5.0, 15.0	-15.0, 55.0
Week 52		
n	12	11
Mean (SD)	74.17 (14.43)	84.55 (9.86)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	85.00
25th, 75th Percentile	62.50, 85.00	80.00, 90.00
Min, Max	50.0, 100.0	65.0, 100.0
Change from baseline to Week 52 ^a		
n	11	11
Mean (SD)	-5.91 (16.25)	8.64 (25.89)
Median	-5.00	5.00
25th, 75th Percentile	-10.00, 5.00	-10.00, 15.00
Min, Max	-45.0, 15.0	-20.0, 70.0
Difference in change from baseline (95%CI)		14.55 (-4.68, 33.77)
P-value ^b		0.1302

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.65 (-0.22, 1.50)
P-value for interaction term, treatment * [Age at Baseline]		0.2272

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	22	16
Mean (SD)	71.59 (18.73)	70.31 (18.02)
Median	75.00	72.50
25th, 75th Percentile	60.00, 90.00	57.50, 85.00
Min, Max	35.0, 100.0	40.0, 100.0
Week 26		
n	27	19
Mean (SD)	71.30 (18.99)	71.32 (15.89)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	55.00, 90.00	60.00, 80.00
Min, Max	40.0, 100.0	30.0, 95.0
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	-2.25 (17.73)	-0.33 (13.29)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 12.50	-10.00, 10.00
Min, Max	-40.0, 20.0	-25.0, 20.0
Week 52		
n	31	23
Mean (SD)	65.48 (18.09)	71.30 (20.52)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.00	80.00
25th, 75th Percentile	50.00, 80.00	55.00, 80.00
Min, Max	15.0, 95.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	21	14
Mean (SD)	-7.14 (24.47)	-0.36 (25.45)
Median	-10.00	2.50
25th, 75th Percentile	-15.00, 5.00	-5.00, 15.00
Min, Max	-65.0, 40.0	-70.0, 40.0
Difference in change from baseline (95%CI)		6.79 (-10.67, 24.24)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.4346
Hedges'g (95% CI) ^c		0.27 (-0.41, 0.94)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	13	12
Mean (SD)	70.38 (21.55)	77.50 (21.90)
Median	70.00	80.00
25th, 75th Percentile	50.00, 90.00	72.50, 90.00
Min, Max	40.0, 100.0	15.0, 100.0
Week 26		
n	12	11
Mean (SD)	76.67 (18.26)	73.64 (15.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	75.00
25th, 75th Percentile	60.00, 90.00	65.00, 90.00
Min, Max	50.0, 100.0	50.0, 95.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	4.17 (5.97)	-2.27 (22.18)
Median	2.50	-10.00
25th, 75th Percentile	0.00, 10.00	-15.00, 5.00
Min, Max	-5.0, 15.0	-30.0, 55.0
Week 52		
n	12	11
Mean (SD)	67.92 (21.69)	75.91 (14.29)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	80.00
25th, 75th Percentile	55.00, 80.00	65.00, 90.00
Min, Max	20.0, 100.0	50.0, 90.0
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	-2.92 (22.41)	0.00 (27.20)
Median	-2.50	-5.00
25th, 75th Percentile	-10.00, 7.50	-20.00, 10.00
Min, Max	-50.0, 40.0	-30.0, 70.0
Difference in change from baseline (95%CI)		2.92 (-18.62, 24.45)
P-value ^b		0.7810

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.11 (-0.71, 0.93)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.7748

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	4	8
Mean (SD)	62.50 (9.57)	76.25 (17.06)
Median	60.00	75.00
25th, 75th Percentile	55.00, 70.00	65.00, 90.00
Min, Max	55.0, 75.0	50.0, 100.0
Week 26		
n	6	7
Mean (SD)	63.33 (21.13)	77.86 (13.80)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	57.50	75.00
25th, 75th Percentile	45.00, 85.00	65.00, 90.00
Min, Max	45.0, 90.0	60.0, 95.0
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	0.00 (17.80)	7.50 (9.35)
Median	2.50	7.50
25th, 75th Percentile	-15.00, 15.00	0.00, 15.00
Min, Max	-20.0, 15.0	-5.0, 20.0
Week 52		
n	6	9
Mean (SD)	65.00 (14.49)	77.22 (16.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	50.00, 75.00	75.00, 90.00
Min, Max	45.0, 80.0	45.0, 95.0
Change from baseline to Week 52 ^a		
n	4	5
Mean (SD)	3.75 (11.09)	8.00 (13.04)
Median	5.00	10.00
25th, 75th Percentile	-5.00, 12.50	-5.00, 15.00
Min, Max	-10.0, 15.0	-5.0, 25.0
Difference in change from baseline (95%CI)		4.25 (-15.17, 23.67)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6207
Hedges'g (95% CI) ^c		0.31 (-1.03, 1.62)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	14	8
Mean (SD)	72.14 (19.97)	71.25 (18.08)
Median	72.50	77.50
25th, 75th Percentile	60.00, 90.00	57.50, 82.50
Min, Max	35.0, 100.0	40.0, 95.0
Week 26		
n	13	8
Mean (SD)	81.54 (15.19)	66.25 (18.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	67.50
25th, 75th Percentile	75.00, 90.00	60.00, 77.50
Min, Max	50.0, 100.0	30.0, 90.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	4.09 (6.25)	-5.00 (14.64)
Median	0.00	-5.00
25th, 75th Percentile	0.00, 10.00	-17.50, 7.50
Min, Max	-5.0, 15.0	-25.0, 15.0
Week 52		
n	16	9
Mean (SD)	64.38 (21.05)	63.89 (23.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	67.50	65.00
25th, 75th Percentile	47.50, 77.50	55.00, 80.00
Min, Max	20.0, 95.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-6.15 (22.65)	-6.88 (32.62)
Median	-10.00	0.00
25th, 75th Percentile	-20.00, 5.00	-22.50, 10.00
Min, Max	-45.0, 40.0	-70.0, 40.0
Difference in change from baseline (95%CI)		-0.72 (-25.89, 24.45)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.9528
Hedges'g (95% CI) ^c		-0.03 (-0.91, 0.86)

Max, maximum; Min, minimum; SD, standard deviation.

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^b Two-sided p-value.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	11	8
Mean (SD)	70.91 (22.23)	67.50 (26.86)
Median	75.00	77.50
25th, 75th Percentile	40.00, 85.00	52.50, 82.50
Min, Max	40.0, 100.0	15.0, 100.0
Week 26		
n	14	11
Mean (SD)	69.29 (18.17)	72.27 (14.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	55.00, 85.00	60.00, 85.00
Min, Max	40.0, 100.0	50.0, 90.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	-2.27 (16.64)	0.63 (24.99)
Median	5.00	-2.50
25th, 75th Percentile	-10.00, 10.00	-12.50, 5.00
Min, Max	-40.0, 15.0	-30.0, 55.0
Week 52		
n	16	12
Mean (SD)	65.31 (18.84)	72.50 (16.17)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.00	80.00
25th, 75th Percentile	55.00, 80.00	60.00, 82.50
Min, Max	15.0, 90.0	45.0, 95.0
Change from baseline to Week 52 ^a		
n	11	8
Mean (SD)	-8.18 (30.02)	1.25 (30.79)
Median	-5.00	-5.00
25th, 75th Percentile	-15.00, 5.00	-17.50, 7.50
Min, Max	-65.0, 40.0	-30.0, 70.0
Difference in change from baseline (95%CI)		9.43 (-20.31, 39.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.5125
Hedges'g (95% CI) ^c		0.30 (-0.62, 1.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	6	4
Mean (SD)	75.00 (20.74)	83.75 (10.31)
Median	85.00	85.00
25th, 75th Percentile	60.00, 90.00	77.50, 90.00
Min, Max	40.0, 90.0	70.0, 95.0
Week 26		
n	6	4
Mean (SD)	72.50 (21.39)	73.75 (18.43)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	75.00
25th, 75th Percentile	50.00, 90.00	62.50, 85.00
Min, Max	50.0, 95.0	50.0, 95.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	-2.50 (21.39)	-10.00 (8.16)
Median	0.00	-10.00
25th, 75th Percentile	-10.00, 15.00	-15.00, -5.00
Min, Max	-40.0, 20.0	-20.0, 0.0
Week 52		
n	5	4
Mean (SD)	76.00 (19.17)	83.75 (13.77)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	82.50
25th, 75th Percentile	65.00, 85.00	72.50, 95.00
Min, Max	50.0, 100.0	70.0, 100.0
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	-6.00 (20.43)	0.00 (10.80)
Median	5.00	-2.50
25th, 75th Percentile	-10.00, 5.00	-7.50, 7.50
Min, Max	-40.0, 10.0	-10.0, 15.0
Difference in change from baseline (95%CI)		6.00 (-20.95, 32.95)
P-value ^b		0.6148

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.5
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: Social Functioning Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.31 (-1.02, 1.63)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.9416

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	13	9
Mean (SD)	67.31 (17.87)	72.22 (15.63)
Median	65.00	80.00
25th, 75th Percentile	55.00, 80.00	60.00, 85.00
Min, Max	40.0, 100.0	40.0, 85.0
Week 26		
n	14	10
Mean (SD)	67.14 (18.05)	72.50 (12.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	72.50
25th, 75th Percentile	50.00, 85.00	60.00, 80.00
Min, Max	40.0, 95.0	55.0, 90.0
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-1.54 (18.64)	0.00 (10.00)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 10.00	-7.50, 7.50
Min, Max	-40.0, 15.0	-15.0, 15.0
Week 52		
n	14	11
Mean (SD)	57.50 (20.64)	77.73 (17.52)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	57.50	80.00
25th, 75th Percentile	45.00, 75.00	65.00, 95.00
Min, Max	15.0, 90.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-10.00 (26.37)	8.75 (14.82)
Median	-10.00	5.00
25th, 75th Percentile	-20.00, 5.00	-2.50, 15.00
Min, Max	-65.0, 40.0	-5.0, 40.0
Difference in change from baseline (95%CI)		18.75 (-2.91, 40.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0857
Hedges'g (95% CI) ^c		0.79 (-0.15, 1.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	14	8
Mean (SD)	71.43 (21.43)	73.13 (22.67)
Median	77.50	70.00
25th, 75th Percentile	50.00, 90.00	52.50, 97.50
Min, Max	35.0, 95.0	45.0, 100.0
Week 26		
n	12	10
Mean (SD)	77.92 (17.12)	71.00 (20.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.005.006_qs_sum_ovr_ped_self_soc_bhagv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	82.50	77.50
25th, 75th Percentile	65.00, 90.00	55.00, 90.00
Min, Max	50.0, 100.0	30.0, 95.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	2.73 (6.47)	-4.38 (17.41)
Median	0.00	-7.50
25th, 75th Percentile	0.00, 5.00	-20.00, 12.50
Min, Max	-10.0, 15.0	-25.0, 20.0
Week 52		
n	15	9
Mean (SD)	68.67 (18.17)	67.22 (24.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	65.00, 80.00	55.00, 80.00
Min, Max	20.0, 100.0	25.0, 95.0
Change from baseline to Week 52 ^a		
n	14	7
Mean (SD)	-2.50 (22.25)	-10.00 (30.14)
Median	-5.00	-5.00
25th, 75th Percentile	-10.00, 10.00	-20.00, 10.00
Min, Max	-45.0, 40.0	-70.0, 25.0
Difference in change from baseline (95%CI)		-7.50 (-31.73, 16.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.5249
Hedges'g (95% CI) ^c		-0.29 (-1.20, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	8	11
Mean (SD)	76.88 (19.63)	74.55 (22.19)
Median	77.50	80.00
25th, 75th Percentile	67.50, 92.50	70.00, 85.00
Min, Max	40.0, 100.0	15.0, 95.0
Week 26		
n	13	10
Mean (SD)	74.62 (20.46)	73.00 (13.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	72.50
25th, 75th Percentile	60.00, 90.00	65.00, 85.00
Min, Max	45.0, 100.0	50.0, 95.0
Change from baseline to Week 26 ^a		
n	8	10
Mean (SD)	-0.63 (16.78)	0.50 (22.29)
Median	0.00	0.00
25th, 75th Percentile	-15.00, 15.00	-10.00, 5.00
Min, Max	-25.0, 20.0	-30.0, 55.0
Week 52		
n	14	14
Mean (SD)	72.14 (15.78)	72.50 (15.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	77.50
25th, 75th Percentile	60.00, 85.00	60.00, 85.00
Min, Max	50.0, 95.0	45.0, 90.0
Change from baseline to Week 52 ^a		
n	7	10
Mean (SD)	-4.29 (22.99)	-0.50 (28.91)
Median	-5.00	-7.50
25th, 75th Percentile	-10.00, 15.00	-20.00, 10.00
Min, Max	-50.0, 20.0	-30.0, 70.0
Difference in change from baseline (95%CI)		3.79 (-24.26, 31.83)
P-value ^b		0.7775

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.13 (-0.83, 1.10)
P-value for interaction term, treatment * [Baseline AGV]		0.2697

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	24	22
Mean (SD)	69.58 (18.76)	73.64 (20.77)
Median	72.50	80.00
25th, 75th Percentile	57.50, 82.50	60.00, 85.00
Min, Max	40.0, 100.0	15.0, 100.0
Week 26		
n	27	25
Mean (SD)	72.78 (18.31)	72.40 (15.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	55.00, 90.00	65.00, 80.00
Min, Max	40.0, 100.0	30.0, 95.0
Change from baseline to Week 26 ^a		
n	22	21
Mean (SD)	1.59 (14.59)	-2.14 (17.93)
Median	5.00	-5.00
25th, 75th Percentile	0.00, 10.00	-10.00, 5.00
Min, Max	-40.0, 20.0	-30.0, 55.0
Week 52		
n	30	27
Mean (SD)	67.00 (17.20)	72.96 (20.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	60.00, 80.00	55.00, 90.00
Min, Max	15.0, 90.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	23	21
Mean (SD)	-4.35 (22.17)	-0.48 (27.47)
Median	-5.00	-5.00
25th, 75th Percentile	-15.00, 5.00	-10.00, 10.00
Min, Max	-65.0, 40.0	-70.0, 70.0
Difference in change from baseline (95%CI)		3.87 (-11.26, 19.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6082
Hedges'g (95% CI) ^c		0.15 (-0.44, 0.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported PedsQL : Social Functioning Score		
Baseline		
n	11	6
Mean (SD)	74.55 (21.62)	72.50 (16.96)
Median	75.00	70.00
25th, 75th Percentile	55.00, 90.00	70.00, 85.00
Min, Max	35.0, 100.0	45.0, 95.0
Week 26		
n	12	5
Mean (SD)	73.33 (20.38)	71.00 (17.82)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.50	75.00
25th, 75th Percentile	50.00, 90.00	55.00, 85.00
Min, Max	45.0, 100.0	50.0, 90.0
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	-3.00 (15.13)	3.00 (14.83)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 5.00	0.00, 10.00
Min, Max	-40.0, 15.0	-20.0, 20.0
Week 52		
n	13	7
Mean (SD)	64.23 (23.08)	72.14 (13.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.00	70.00
25th, 75th Percentile	50.00, 80.00	60.00, 85.00
Min, Max	20.0, 100.0	55.0, 90.0
Change from baseline to Week 52 ^a		
n	10	4
Mean (SD)	-8.50 (27.29)	1.25 (15.48)
Median	-10.00	5.00
25th, 75th Percentile	-30.00, 10.00	-10.00, 12.50
Min, Max	-50.0, 40.0	-20.0, 15.0
Difference in change from baseline (95%CI)		9.75 (-22.31, 41.81)
P-value ^b		0.5200

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.5.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: Social Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.37 (-0.81, 1.53)
P-value for interaction term, treatment * [Ethnicity]		0.7230

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Social functioning score is the mean score (sum of items scored/number of items answered in social functioning domain).

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Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	20	15
Mean (SD)	74.88 (15.56)	65.67 (21.87)
Median	75.00	70.00
25th, 75th Percentile	65.00, 90.00	50.00, 80.00
Min, Max	40.0, 95.0	15.0, 95.0
Week 26		
n	19	14
Mean (SD)	72.63 (18.44)	67.77 (22.82)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	72.50
25th, 75th Percentile	60.00, 85.00	55.00, 85.00
Min, Max	40.0, 100.0	5.0, 93.8
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-2.50 (17.72)	2.41 (17.94)
Median	-1.25	2.50
25th, 75th Percentile	-10.00, 8.75	-10.00, 10.00
Min, Max	-30.0, 35.0	-25.0, 43.8
Week 52		
n	23	17
Mean (SD)	73.48 (16.06)	68.53 (14.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	65.00
25th, 75th Percentile	65.00, 80.00	55.00, 80.00
Min, Max	25.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	-1.81 (22.74)	1.43 (18.34)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-65.0, 40.0	-30.0, 40.0
Difference in change from baseline (95%CI)		3.23 (-12.01, 18.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6679
Hedges'g (95% CI) ^c		0.15 (-0.55, 0.85)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	15	13
Mean (SD)	77.33 (19.07)	71.54 (14.05)
Median	85.00	70.00
25th, 75th Percentile	55.00, 95.00	65.00, 85.00
Min, Max	50.0, 100.0	40.0, 90.0
Week 26		
n	20	16
Mean (SD)	78.50 (17.33)	73.13 (15.04)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	82.50	70.00
25th, 75th Percentile	70.00, 90.00	60.00, 90.00
Min, Max	40.0, 100.0	50.0, 95.0
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	2.33 (13.07)	1.67 (11.74)
Median	-5.00	0.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 7.50
Min, Max	-15.0, 25.0	-15.0, 25.0
Week 52		
n	20	17
Mean (SD)	71.25 (13.36)	80.37 (14.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	85.00
25th, 75th Percentile	60.00, 80.00	70.00, 90.00
Min, Max	50.0, 100.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	15	11
Mean (SD)	-5.33 (13.69)	3.64 (17.90)
Median	-5.00	5.00
25th, 75th Percentile	-15.00, 5.00	-5.00, 20.00
Min, Max	-35.0, 15.0	-35.0, 30.0
Difference in change from baseline (95%CI)		8.97 (-3.80, 21.74)
P-value ^b		0.1600

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.1
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Sex: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.56 (-0.24, 1.35)
P-value for interaction term, treatment * [Sex]		0.5688

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported PedsQL : School Functioning Score		
Week 26		
n	5	4
Mean (SD)	67.00 (20.19)	73.75 (22.13)
Median	65.00	75.00
25th, 75th Percentile	50.00, 85.00	55.00, 92.50
Min, Max	45.0, 90.0	50.0, 95.0
Week 52		
n	8	8
Mean (SD)	66.88 (11.63)	88.91 (12.35)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	62.50	90.00
25th, 75th Percentile	57.50, 77.50	83.13, 100.00
Min, Max	55.0, 85.0	65.0, 100.0
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	23	16
Mean (SD)	75.92 (16.02)	71.56 (13.87)
Median	75.00	75.00
25th, 75th Percentile	60.00, 90.00	65.00, 82.50
Min, Max	50.0, 100.0	40.0, 90.0
Week 26		
n	23	15
Mean (SD)	79.35 (15.83)	71.25 (13.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	70.00
25th, 75th Percentile	75.00, 90.00	60.00, 80.00
Min, Max	45.0, 100.0	55.0, 93.8
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	2.44 (15.42)	-0.08 (16.88)
Median	-3.13	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 5.00
Min, Max	-25.0, 35.0	-25.0, 43.8
Week 52		
n	23	15
Mean (SD)	75.22 (12.57)	71.33 (15.29)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.00	75.00
25th, 75th Percentile	65.00, 85.00	60.00, 80.00
Min, Max	50.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	22	14
Mean (SD)	-1.88 (16.58)	0.71 (12.07)
Median	-0.63	2.50
25th, 75th Percentile	-10.00, 10.00	-5.00, 10.00
Min, Max	-35.0, 40.0	-30.0, 20.0
Difference in change from baseline (95%CI)		2.59 (-7.84, 13.02)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.6.2
 Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
 Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6173
Hedges'g (95% CI) ^c		0.17 (-0.50, 0.84)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	12	12
Mean (SD)	75.94 (19.31)	64.17 (23.44)
Median	83.13	67.50
25th, 75th Percentile	57.50, 90.00	52.50, 80.00
Min, Max	40.0, 100.0	15.0, 95.0
Week 26		
n	11	11
Mean (SD)	71.82 (20.40)	68.64 (25.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	60.00, 90.00	60.00, 85.00
Min, Max	40.0, 100.0	5.0, 95.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	-6.13 (15.28)	5.00 (12.45)
Median	-2.50	5.00
25th, 75th Percentile	-15.00, 5.00	-10.00, 15.00
Min, Max	-30.0, 15.0	-10.0, 25.0
Week 52		
n	12	11
Mean (SD)	70.83 (19.64)	68.18 (11.68)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	65.00
25th, 75th Percentile	62.50, 80.00	55.00, 80.00
Min, Max	25.0, 100.0	55.0, 85.0
Change from baseline to Week 52 ^a		
n	11	11
Mean (SD)	-6.48 (23.64)	4.55 (23.71)
Median	0.00	-5.00
25th, 75th Percentile	-20.00, 5.00	-15.00, 30.00
Min, Max	-65.0, 25.0	-35.0, 40.0
Difference in change from baseline (95%CI)		11.02 (-10.04, 32.08)
P-value ^b		0.2879

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.2
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Age at Baseline: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.45 (-0.40, 1.29)
P-value for interaction term, treatment * [Age at Baseline]		0.4129

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.006.002_qs_sum_ovr_ped_self_sch_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	22	16
Mean (SD)	76.65 (14.85)	64.38 (20.48)
Median	77.50	67.50
25th, 75th Percentile	65.00, 90.00	55.00, 77.50
Min, Max	50.0, 100.0	15.0, 90.0
Week 26		
n	27	19
Mean (SD)	75.37 (17.92)	67.57 (21.92)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	65.00
25th, 75th Percentile	65.00, 90.00	55.00, 90.00
Min, Max	40.0, 100.0	5.0, 95.0
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	0.44 (16.93)	1.92 (16.97)
Median	-0.63	0.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-30.0, 35.0	-25.0, 43.8
Week 52		
n	31	23
Mean (SD)	72.10 (15.53)	73.97 (17.34)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	65.00, 80.00	60.00, 85.00
Min, Max	25.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	21	14
Mean (SD)	-3.87 (20.48)	2.86 (17.06)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-5.00, 10.00
Min, Max	-65.0, 40.0	-30.0, 40.0
Difference in change from baseline (95%CI)		6.73 (-6.76, 20.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.3175
Hedges'g (95% CI) ^c		0.34 (-0.34, 1.02)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	13	12
Mean (SD)	74.71 (20.59)	73.75 (14.79)
Median	81.25	72.50
25th, 75th Percentile	55.00, 95.00	65.00, 85.00
Min, Max	40.0, 100.0	40.0, 95.0
Week 26		
n	12	11
Mean (SD)	76.25 (18.60)	75.91 (11.14)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	82.50	75.00
25th, 75th Percentile	65.00, 90.00	65.00, 85.00
Min, Max	40.0, 95.0	60.0, 95.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	-1.35 (13.91)	2.27 (12.92)
Median	-5.00	0.00
25th, 75th Percentile	-7.50, 6.88	-10.00, 10.00
Min, Max	-30.0, 25.0	-15.0, 25.0
Week 52		
n	12	11
Mean (SD)	73.33 (13.03)	75.45 (11.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	62.50, 82.50	65.00, 85.00
Min, Max	55.0, 100.0	55.0, 90.0
Change from baseline to Week 52 ^a		
n	12	11
Mean (SD)	-2.60 (16.84)	1.82 (19.53)
Median	-0.63	5.00
25th, 75th Percentile	-12.50, 7.50	-15.00, 20.00
Min, Max	-35.0, 25.0	-35.0, 30.0
Difference in change from baseline (95%CI)		4.42 (-11.35, 20.20)
P-value ^b		0.5661

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.3
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Tanner Stage: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.23 (-0.59, 1.05)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.8219

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	4	8
Mean (SD)	70.00 (20.41)	73.75 (9.54)
Median	70.00	72.50
25th, 75th Percentile	52.50, 87.50	65.00, 80.00
Min, Max	50.0, 90.0	65.0, 90.0
Week 26		
n	6	7
Mean (SD)	63.33 (19.41)	70.71 (11.34)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	60.00	70.00
25th, 75th Percentile	45.00, 80.00	60.00, 75.00
Min, Max	45.0, 90.0	55.0, 90.0
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	1.25 (11.09)	-1.67 (10.33)
Median	0.00	-2.50
25th, 75th Percentile	-7.50, 10.00	-10.00, 10.00
Min, Max	-10.0, 15.0	-15.0, 10.0
Week 52		
n	6	9
Mean (SD)	71.67 (16.93)	79.58 (15.31)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	55.00, 90.00	75.00, 85.00
Min, Max	55.0, 90.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	4	5
Mean (SD)	10.00 (21.21)	0.00 (12.75)
Median	5.00	-5.00
25th, 75th Percentile	-2.50, 22.50	-10.00, 5.00
Min, Max	-10.0, 40.0	-10.0, 20.0
Difference in change from baseline (95%CI)		-10.00 (-36.81, 16.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.4071
Hedges'g (95% CI) ^c		-0.53 (-1.85, 0.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	14	8
Mean (SD)	75.89 (17.58)	56.25 (21.00)
Median	81.25	62.50
25th, 75th Percentile	60.00, 90.00	45.00, 70.00
Min, Max	40.0, 100.0	15.0, 80.0
Week 26		
n	13	8
Mean (SD)	75.38 (20.56)	59.84 (25.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	62.50
25th, 75th Percentile	60.00, 90.00	55.00, 72.50
Min, Max	40.0, 100.0	5.0, 93.8
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	-3.86 (16.47)	3.59 (21.67)
Median	0.00	0.00
25th, 75th Percentile	-15.00, 5.00	-10.00, 15.00
Min, Max	-30.0, 25.0	-25.0, 43.8
Week 52		
n	16	9
Mean (SD)	71.25 (16.68)	63.33 (11.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	65.00
25th, 75th Percentile	65.00, 80.00	55.00, 70.00
Min, Max	25.0, 100.0	50.0, 85.0
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-5.19 (22.23)	6.88 (22.51)
Median	-1.25	2.50
25th, 75th Percentile	-5.00, 5.00	-5.00, 25.00
Min, Max	-65.0, 25.0	-30.0, 40.0
Difference in change from baseline (95%CI)		12.07 (-8.94, 33.07)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2439
Hedges'g (95% CI) ^c		0.52 (-0.38, 1.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	11	8
Mean (SD)	75.91 (16.25)	71.25 (14.82)
Median	75.00	72.50
25th, 75th Percentile	60.00, 95.00	67.50, 80.00
Min, Max	50.0, 95.0	40.0, 90.0
Week 26		
n	14	11
Mean (SD)	80.36 (12.48)	75.91 (15.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	80.00
25th, 75th Percentile	75.00, 90.00	60.00, 90.00
Min, Max	45.0, 95.0	50.0, 95.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	4.09 (14.46)	3.75 (14.08)
Median	-5.00	2.50
25th, 75th Percentile	-5.00, 20.00	-7.50, 15.00
Min, Max	-15.0, 25.0	-15.0, 25.0
Week 52		
n	16	12
Mean (SD)	70.94 (12.81)	79.58 (15.59)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	82.50
25th, 75th Percentile	62.50, 80.00	70.00, 90.00
Min, Max	50.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	11	8
Mean (SD)	-6.36 (15.02)	2.50 (10.35)
Median	-10.00	5.00
25th, 75th Percentile	-15.00, 10.00	-5.00, 10.00
Min, Max	-35.0, 10.0	-15.0, 15.0
Difference in change from baseline (95%CI)		8.86 (-4.17, 21.90)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.1695
Hedges'g (95% CI) ^c		0.64 (-0.31, 1.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	6	4
Mean (SD)	80.00 (17.89)	76.25 (27.80)
Median	82.50	87.50
25th, 75th Percentile	65.00, 95.00	60.00, 92.50
Min, Max	55.0, 100.0	35.0, 95.0
Week 26		
n	6	4
Mean (SD)	77.50 (19.69)	77.50 (19.36)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.50	82.50
25th, 75th Percentile	70.00, 95.00	65.00, 90.00
Min, Max	45.0, 100.0	50.0, 95.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	-2.50 (20.19)	1.25 (11.09)
Median	-7.50	0.00
25th, 75th Percentile	-10.00, 0.00	-7.50, 10.00
Min, Max	-25.0, 35.0	-10.0, 15.0
Week 52		
n	5	4
Mean (SD)	82.00 (11.51)	72.50 (15.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	72.50
25th, 75th Percentile	75.00, 85.00	60.00, 85.00
Min, Max	70.0, 100.0	55.0, 90.0
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	-3.00 (16.81)	-3.75 (27.80)
Median	0.00	-5.00
25th, 75th Percentile	-15.00, 10.00	-25.00, 17.50
Min, Max	-25.0, 15.0	-35.0, 30.0
Difference in change from baseline (95%CI)		-0.75 (-35.96, 34.46)
P-value ^b		0.9612

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.5
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline Height Z-score: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.03 (-1.34, 1.29)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.4867

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	13	9
Mean (SD)	73.08 (17.50)	60.00 (21.36)
Median	70.00	65.00
25th, 75th Percentile	60.00, 85.00	60.00, 75.00
Min, Max	50.0, 95.0	15.0, 80.0
Week 26		
n	14	10
Mean (SD)	69.64 (19.26)	60.50 (24.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.50	60.00
25th, 75th Percentile	45.00, 90.00	55.00, 80.00
Min, Max	40.0, 90.0	5.0, 95.0
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-1.54 (14.34)	-2.50 (10.35)
Median	0.00	-5.00
25th, 75th Percentile	-5.00, 5.00	-10.00, 5.00
Min, Max	-30.0, 20.0	-15.0, 15.0
Week 52		
n	14	11
Mean (SD)	71.43 (14.47)	72.84 (14.45)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	70.00
25th, 75th Percentile	60.00, 85.00	60.00, 81.25
Min, Max	50.0, 95.0	55.0, 100.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-0.83 (17.17)	6.88 (18.11)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 5.00	-5.00, 17.50
Min, Max	-25.0, 40.0	-10.0, 40.0
Difference in change from baseline (95%CI)		7.71 (-9.11, 24.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.3484
Hedges'g (95% CI) ^c		0.42 (-0.49, 1.32)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	14	8
Mean (SD)	77.95 (18.17)	75.00 (14.14)
Median	78.13	77.50
25th, 75th Percentile	70.00, 90.00	65.00, 87.50
Min, Max	40.0, 100.0	50.0, 90.0
Week 26		
n	12	10
Mean (SD)	79.58 (17.38)	75.38 (16.07)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	82.50	77.50
25th, 75th Percentile	70.00, 92.50	60.00, 90.00
Min, Max	40.0, 100.0	50.0, 93.8
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	0.34 (16.43)	1.72 (19.60)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 10.00	-5.00, 5.00
Min, Max	-30.0, 25.0	-25.0, 43.8
Week 52		
n	15	9
Mean (SD)	71.67 (17.90)	74.44 (20.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	65.00, 80.00	55.00, 90.00
Min, Max	25.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	14	7
Mean (SD)	-5.45 (21.95)	-3.57 (13.45)
Median	-0.63	0.00
25th, 75th Percentile	-15.00, 10.00	-10.00, 5.00
Min, Max	-65.0, 25.0	-30.0, 10.0
Difference in change from baseline (95%CI)		1.88 (-17.18, 20.93)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8390
Hedges'g (95% CI) ^c		0.09 (-0.82, 1.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	8	11
Mean (SD)	77.03 (15.15)	70.45 (17.95)
Median	80.63	70.00
25th, 75th Percentile	62.50, 90.00	65.00, 85.00
Min, Max	55.0, 95.0	40.0, 95.0
Week 26		
n	13	10
Mean (SD)	78.46 (16.38)	76.00 (11.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.07.002.006.006_qs_sum_ovr_ped_self_sch_bhagv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	80.00	72.50
25th, 75th Percentile	75.00, 85.00	65.00, 85.00
Min, Max	45.0, 100.0	65.0, 95.0
Change from baseline to Week 26 ^a		
n	8	10
Mean (SD)	1.09 (18.49)	6.00 (14.68)
Median	-7.50	7.50
25th, 75th Percentile	-10.00, 11.88	-10.00, 20.00
Min, Max	-15.0, 35.0	-15.0, 25.0
Week 52		
n	14	14
Mean (SD)	74.29 (11.91)	75.71 (13.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.50	80.00
25th, 75th Percentile	65.00, 80.00	65.00, 85.00
Min, Max	55.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	7	10
Mean (SD)	-3.75 (17.69)	3.00 (20.58)
Median	-1.25	10.00
25th, 75th Percentile	-15.00, 10.00	-15.00, 20.00
Min, Max	-35.0, 15.0	-35.0, 30.0
Difference in change from baseline (95%CI)		6.75 (-13.70, 27.20)
P-value ^b		0.4926

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Table 14.2.7.2.6.6
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Baseline AGV Category: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.33 (-0.65, 1.30)
P-value for interaction term, treatment * [Baseline AGV]		0.8806

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	24	22
Mean (SD)	74.22 (16.68)	64.09 (18.56)
Median	75.00	65.00
25th, 75th Percentile	60.00, 90.00	60.00, 75.00
Min, Max	40.0, 100.0	15.0, 95.0
Week 26		
n	27	25
Mean (SD)	76.85 (19.02)	68.75 (19.84)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

School functioning score is the mean score (sum of items scored/number of items answered in school functioning domain).

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	85.00	65.00
25th, 75th Percentile	70.00, 90.00	60.00, 85.00
Min, Max	40.0, 100.0	5.0, 95.0
Change from baseline to Week 26 ^a		
n	22	21
Mean (SD)	1.53 (16.90)	3.99 (16.02)
Median	2.50	5.00
25th, 75th Percentile	-10.00, 10.00	-10.00, 10.00
Min, Max	-30.0, 35.0	-25.0, 43.8
Week 52		
n	30	27
Mean (SD)	72.17 (15.68)	72.45 (15.30)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

A higher score reflects a higher quality of life.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	75.00
25th, 75th Percentile	65.00, 80.00	60.00, 85.00
Min, Max	25.0, 100.0	50.0, 100.0
Change from baseline to Week 52 ^a		
n	23	21
Mean (SD)	-2.45 (17.70)	3.33 (18.80)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-5.00, 15.00
Min, Max	-65.0, 25.0	-35.0, 40.0
Difference in change from baseline (95%CI)		5.78 (-5.32, 16.88)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2995
Hedges'g (95% CI) ^c		0.31 (-0.29, 0.90)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported PedsQL : School Functioning Score		
Baseline		
n	11	6
Mean (SD)	79.66 (17.68)	84.17 (5.85)
Median	81.25	85.00
25th, 75th Percentile	60.00, 95.00	80.00, 90.00
Min, Max	50.0, 100.0	75.0, 90.0
Week 26		
n	12	5
Mean (SD)	72.92 (15.44)	80.00 (10.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.50	80.00
25th, 75th Percentile	62.50, 82.50	70.00, 90.00
Min, Max	45.0, 95.0	70.0, 90.0
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	-4.13 (12.39)	-6.00 (6.52)
Median	-5.00	-5.00
25th, 75th Percentile	-10.00, -1.25	-10.00, 0.00
Min, Max	-25.0, 25.0	-15.0, 0.0
Week 52		
n	13	7
Mean (SD)	73.08 (12.84)	82.14 (14.96)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	70.00	80.00
25th, 75th Percentile	65.00, 80.00	65.00, 100.00
Min, Max	55.0, 100.0	65.0, 100.0
Change from baseline to Week 52 ^a		
n	10	4
Mean (SD)	-5.63 (22.51)	-2.50 (11.90)
Median	-10.00	-2.50
25th, 75th Percentile	-25.00, 10.00	-12.50, 7.50
Min, Max	-35.0, 40.0	-15.0, 10.0
Difference in change from baseline (95%CI)		3.13 (-23.15, 29.40)
P-value ^b		0.7999

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.7.2.6.7
Self-Reported Pediatric Quality of Life Inventory (PedsQL) Over Time by Ethnicity: School Functioning Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.14 (-1.02, 1.30)
P-value for interaction term, treatment * [Ethnicity]		0.8318

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	33	31
Mean (SD)	59.63 (18.87)	57.56 (18.63)
Median	60.77	59.72
25th, 75th Percentile	49.31, 71.18	46.93, 68.40
Min, Max	20.8, 92.4	14.6, 91.3
Week 26		
n	31	28
Mean (SD)	59.41 (16.69)	54.91 (18.97)
Median	63.54	55.98

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	44.10, 70.49	45.49, 66.39
Min, Max	25.4, 92.7	7.6, 87.1
Change from baseline to Week 26 ^a		
n	31	28
Mean (SD)	-1.23 (12.72)	-1.32 (14.90)
Median	-0.69	-0.75
25th, 75th Percentile	-7.69, 5.90	-7.64, 9.53
Min, Max	-27.4, 23.3	-55.6, 17.0
Week 52		
n	32	30
Mean (SD)	59.91 (20.83)	55.48 (19.60)
Median	64.90	57.99

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.01, 75.52	48.96, 64.58
Min, Max	10.1, 88.5	1.2, 90.6
Change from baseline to Week 52 ^a		
n	32	30
Mean (SD)	-0.35 (15.80)	-1.77 (17.63)
Median	-0.72	-2.26
25th, 75th Percentile	-13.37, 11.25	-9.28, 10.76
Min, Max	-29.5, 38.9	-62.0, 34.4
Difference in change from baseline (95%CI)		-1.42 (-9.92, 7.07)
P-value ^b		0.7387
Hedges'g (95% CI) ^c		-0.08 (-0.58, 0.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	28	29
Mean (SD)	50.04 (23.66)	56.40 (16.27)
Median	49.14	54.52
25th, 75th Percentile	30.21, 68.06	47.57, 68.40
Min, Max	13.5, 91.0	22.6, 97.2
Week 26		
n	28	27
Mean (SD)	54.30 (21.45)	63.30 (16.15)
Median	60.42	64.93

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	36.81, 70.49	51.04, 72.57
Min, Max	8.7, 91.3	34.0, 94.8
Change from baseline to Week 26 ^a		
n	28	27
Mean (SD)	4.27 (17.26)	5.73 (14.35)
Median	1.22	4.86
25th, 75th Percentile	-3.47, 12.33	-2.43, 15.28
Min, Max	-42.3, 57.3	-36.1, 41.3
Week 52		
n	28	27
Mean (SD)	56.54 (19.95)	58.84 (14.48)
Median	60.42	60.77

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.001.001_qs_sum_ovr_qol_care_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.28, 73.96	47.92, 70.49
Min, Max	15.6, 89.2	27.1, 85.4
Change from baseline to Week 52 ^a		
n	28	27
Mean (SD)	6.50 (13.38)	1.39 (11.05)
Median	3.13	0.69
25th, 75th Percentile	-1.91, 11.64	-4.17, 4.51
Min, Max	-18.4, 50.7	-22.2, 34.0
Difference in change from baseline (95%CI)		-5.11 (-11.76, 1.54)
P-value ^b		0.1293
Hedges'g (95% CI) ^c		-0.41 (-0.94, 0.13)
P-value for interaction term, treatment *[Sex]		0.5032

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	24	31
Mean (SD)	55.70 (20.40)	56.68 (16.61)
Median	56.95	57.64
25th, 75th Percentile	40.11, 69.97	42.71, 67.36
Min, Max	21.5, 91.0	14.6, 86.5
Week 26		
n	24	28
Mean (SD)	53.10 (19.55)	58.63 (19.19)
Median	45.49	58.68

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	36.81, 70.49	49.83, 69.62
Min, Max	25.4, 91.3	7.6, 88.9
Change from baseline to Week 26 ^a		
n	24	28
Mean (SD)	-2.60 (14.73)	2.19 (14.88)
Median	-1.22	2.44
25th, 75th Percentile	-11.29, 6.42	-1.96, 11.98
Min, Max	-42.3, 21.5	-55.6, 27.1
Week 52		
n	23	31
Mean (SD)	59.98 (22.89)	55.78 (18.67)
Median	69.79	60.42

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	41.32, 78.82	44.45, 70.49
Min, Max	10.1, 89.2	1.2, 90.6
Change from baseline to Week 52 ^a		
n	23	31
Mean (SD)	3.58 (14.47)	-0.90 (16.98)
Median	4.16	-1.73
25th, 75th Percentile	-3.47, 14.93	-4.87, 7.29
Min, Max	-22.9, 35.4	-62.0, 34.4
Difference in change from baseline (95%CI)		-4.47 (-13.29, 4.35)
P-value ^b		0.3135
Hedges'g (95% CI) ^c		-0.28 (-0.82, 0.27)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	24	17
Mean (SD)	55.95 (22.83)	53.39 (17.80)
Median	58.86	49.31
25th, 75th Percentile	41.85, 71.36	47.57, 68.40
Min, Max	13.5, 92.4	18.1, 80.6
Week 26		
n	23	16
Mean (SD)	58.24 (19.61)	53.32 (16.73)
Median	63.54	52.43

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.53, 73.96	39.24, 66.67
Min, Max	8.7, 85.1	27.4, 85.4
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	2.22 (13.98)	-0.43 (15.39)
Median	0.00	-2.09
25th, 75th Percentile	-5.21, 9.72	-9.89, 11.81
Min, Max	-27.1, 30.9	-36.1, 22.2
Week 52		
n	24	16
Mean (SD)	55.04 (19.25)	56.09 (17.06)
Median	60.42	56.78

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.29, 67.02	51.04, 66.84
Min, Max	15.6, 88.2	8.3, 81.3
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	-0.91 (15.22)	2.33 (13.91)
Median	-1.56	-0.52
25th, 75th Percentile	-9.90, 8.56	-6.08, 11.70
Min, Max	-29.5, 38.9	-22.2, 34.0
Difference in change from baseline (95%CI)		3.24 (-6.38, 12.85)
P-value ^b		0.4998
Hedges'g (95% CI) ^c		0.22 (-0.42, 0.85)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	13	12
Mean (SD)	53.02 (22.83)	62.94 (18.70)
Median	58.68	64.93
25th, 75th Percentile	35.42, 67.71	47.43, 71.88
Min, Max	13.5, 92.4	36.8, 97.2
Week 26		
n	12	11
Mean (SD)	62.36 (16.98)	68.32 (13.59)
Median	64.24	66.46

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.16, 70.49	55.90, 78.82
Min, Max	22.6, 92.7	49.3, 94.8
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	7.72 (17.00)	5.75 (15.00)
Median	2.61	4.86
25th, 75th Percentile	-1.91, 10.42	-4.52, 13.54
Min, Max	-7.7, 57.3	-10.8, 41.3
Week 52		
n	13	10
Mean (SD)	61.51 (17.93)	62.64 (13.14)
Median	64.24	60.77

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.86, 70.14	52.43, 74.65
Min, Max	26.0, 88.5	45.5, 85.4
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	8.49 (14.65)	-2.51 (8.20)
Median	5.56	-2.95
25th, 75th Percentile	1.04, 10.42	-8.34, 1.73
Min, Max	-7.6, 50.7	-12.5, 13.8
Difference in change from baseline (95%CI)		-11.00 (-21.76, -0.23)
P-value ^b		0.0458
Hedges'g (95% CI) ^c		-0.86 (-1.72, 0.01)
P-value for interaction term, treatment * [Age at Baseline]		0.1846

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	48	48
Mean (SD)	55.43 (21.63)	55.00 (16.51)
Median	58.16	54.69
25th, 75th Percentile	40.11, 71.01	44.82, 67.02
Min, Max	13.5, 92.4	14.6, 86.5
Week 26		
n	46	44
Mean (SD)	56.59 (18.83)	56.75 (18.08)
Median	62.16	58.16

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	38.54, 70.49	45.49, 68.06
Min, Max	21.2, 91.3	7.6, 88.9
Change from baseline to Week 26 ^a		
n	46	44
Mean (SD)	0.66 (16.41)	2.29 (14.86)
Median	0.00	2.79
25th, 75th Percentile	-6.59, 6.94	-3.65, 12.85
Min, Max	-42.3, 57.3	-55.6, 27.1
Week 52		
n	47	47
Mean (SD)	58.39 (20.93)	55.02 (17.37)
Median	62.85	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	41.32, 76.04	46.53, 65.28
Min, Max	10.1, 89.2	1.2, 90.6
Change from baseline to Week 52 ^a		
n	47	47
Mean (SD)	2.62 (16.49)	0.28 (15.82)
Median	0.34	-1.73
25th, 75th Percentile	-5.21, 12.50	-5.21, 10.76
Min, Max	-29.5, 50.7	-62.0, 34.4
Difference in change from baseline (95%CI)		-2.35 (-8.97, 4.27)
P-value ^b		0.4827
Hedges'g (95% CI) ^c		-0.14 (-0.55, 0.26)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	13	12
Mean (SD)	54.49 (22.19)	64.99 (19.22)
Median	58.68	66.49
25th, 75th Percentile	39.59, 70.14	47.75, 77.26
Min, Max	13.5, 92.4	36.8, 97.2
Week 26		
n	13	11
Mean (SD)	58.38 (20.77)	68.12 (15.08)
Median	61.11	67.02

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.63, 66.32	52.78, 80.56
Min, Max	8.7, 92.7	49.3, 94.8
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	3.89 (9.66)	1.55 (15.93)
Median	0.35	-2.44
25th, 75th Percentile	-2.78, 9.37	-8.68, 5.21
Min, Max	-7.7, 27.0	-15.6, 41.3
Week 52		
n	13	10
Mean (SD)	58.15 (18.73)	66.70 (14.00)
Median	60.77	69.97

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.001.003_qs_sum_ovr_qol_care_tot_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.48, 68.75	52.43, 78.82
Min, Max	15.6, 88.5	45.5, 85.4
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	3.66 (7.95)	-2.85 (9.04)
Median	1.39	-0.52
25th, 75th Percentile	-0.70, 7.98	-11.80, 1.73
Min, Max	-8.3, 18.1	-16.0, 13.2
Difference in change from baseline (95%CI)		-6.50 (-13.88, 0.88)
P-value ^b		0.0811
Hedges'g (95% CI) ^c		-0.74 (-1.59, 0.12)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5562

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	10	15
Mean (SD)	52.53 (24.42)	50.97 (13.15)
Median	50.01	49.31
25th, 75th Percentile	34.03, 78.77	42.71, 63.54
Min, Max	19.8, 91.0	22.6, 70.8
Week 26		
n	10	12
Mean (SD)	46.04 (22.91)	61.70 (10.77)
Median	35.94	66.39

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.07, 66.32	55.04, 69.62
Min, Max	21.2, 91.3	41.3, 72.6
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-6.49 (15.33)	10.34 (10.49)
Median	-0.87	11.81
25th, 75th Percentile	-13.89, 1.04	1.22, 18.58
Min, Max	-42.3, 14.9	-4.4, 27.1
Week 52		
n	10	13
Mean (SD)	54.03 (24.51)	55.54 (11.14)
Median	47.40	60.77

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.86, 82.29	52.43, 62.50
Min, Max	27.8, 89.2	29.5, 67.7
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	1.50 (10.59)	5.54 (11.45)
Median	1.56	3.48
25th, 75th Percentile	-1.73, 9.03	-1.73, 11.94
Min, Max	-22.9, 14.9	-8.3, 34.0
Difference in change from baseline (95%CI)		4.04 (-5.66, 13.74)
P-value ^b		0.3961
Hedges'g (95% CI) ^c		0.35 (-0.48, 1.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	24	18
Mean (SD)	56.43 (21.70)	54.88 (13.41)
Median	58.68	52.26
25th, 75th Percentile	39.59, 69.82	46.93, 64.24
Min, Max	13.5, 92.4	32.3, 78.1
Week 26		
n	22	18
Mean (SD)	60.59 (16.11)	48.50 (15.05)
Median	63.37	49.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	44.10, 70.83	40.63, 58.33
Min, Max	32.3, 92.7	7.6, 76.4
Change from baseline to Week 26 ^a		
n	22	18
Mean (SD)	3.03 (15.71)	-6.39 (17.03)
Median	-0.17	-3.12
25th, 75th Percentile	-5.21, 9.37	-9.38, 2.44
Min, Max	-27.1, 57.3	-55.6, 17.0
Week 52		
n	23	18
Mean (SD)	62.05 (20.26)	47.72 (17.32)
Median	64.24	48.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.52, 76.39	41.32, 58.33
Min, Max	10.1, 88.5	1.2, 76.0
Change from baseline to Week 52 ^a		
n	23	18
Mean (SD)	4.89 (14.83)	-7.17 (18.39)
Median	5.56	-3.82
25th, 75th Percentile	-3.47, 12.15	-18.40, 1.39
Min, Max	-29.5, 50.7	-62.0, 19.5
Difference in change from baseline (95%CI)		-12.06 (-22.55, -1.58)
P-value ^b		0.0253
Hedges'g (95% CI) ^c		-0.72 (-1.35, -0.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	19	22
Mean (SD)	52.40 (22.00)	59.66 (21.50)
Median	55.56	61.29
25th, 75th Percentile	35.42, 70.84	41.67, 73.27
Min, Max	13.5, 90.6	14.6, 97.2
Week 26		
n	19	20
Mean (SD)	54.75 (20.56)	63.67 (20.86)
Median	59.38	65.11

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	44.10, 70.14	54.00, 80.73
Min, Max	8.7, 85.1	18.8, 94.8
Change from baseline to Week 26 ^a		
n	19	20
Mean (SD)	2.35 (15.96)	5.19 (12.89)
Median	0.00	4.52
25th, 75th Percentile	-5.56, 18.06	-2.96, 13.02
Min, Max	-27.4, 30.9	-15.6, 41.3
Week 52		
n	19	21
Mean (SD)	54.79 (20.84)	63.04 (18.10)
Median	60.07	65.28

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.98, 72.22	52.78, 74.31
Min, Max	15.6, 88.2	8.3, 90.6
Change from baseline to Week 52 ^a		
n	19	21
Mean (SD)	2.39 (18.45)	2.29 (12.29)
Median	0.00	-1.39
25th, 75th Percentile	-16.67, 17.37	-4.51, 10.41
Min, Max	-26.4, 38.9	-16.0, 34.4
Difference in change from baseline (95%CI)		-0.10 (-10.05, 9.84)
P-value ^b		0.9834
Hedges'g (95% CI) ^c		-0.01 (-0.63, 0.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	8	5
Mean (SD)	61.72 (18.49)	70.97 (14.98)
Median	65.63	65.28
25th, 75th Percentile	58.16, 70.66	64.58, 80.56
Min, Max	20.8, 84.0	53.1, 91.3
Week 26		
n	8	5
Mean (SD)	66.06 (12.81)	71.95 (13.48)
Median	67.54	78.82

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	57.82, 76.91	59.03, 80.56
Min, Max	44.1, 79.9	55.9, 85.4
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	4.34 (10.02)	0.97 (10.35)
Median	1.39	4.86
25th, 75th Percentile	-3.65, 10.94	-8.68, 5.90
Min, Max	-5.9, 23.3	-10.8, 13.5
Week 52		
n	8	5
Mean (SD)	61.46 (13.58)	69.65 (10.83)
Median	63.72	70.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	51.05, 71.88	60.77, 78.82
Min, Max	39.6, 78.8	56.6, 81.3
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-0.26 (12.57)	-1.32 (11.68)
Median	-2.09	-4.51
25th, 75th Percentile	-9.90, 9.38	-7.98, 0.69
Min, Max	-15.6, 18.8	-12.5, 17.7
Difference in change from baseline (95%CI)		-1.06 (-16.44, 14.31)
P-value ^b		0.8820
Hedges'g (95% CI) ^c		-0.08 (-1.20, 1.04)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.1494

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	19	19
Mean (SD)	57.29 (22.43)	59.71 (13.53)
Median	58.33	63.20
25th, 75th Percentile	40.63, 71.18	49.31, 67.36
Min, Max	13.5, 92.4	33.7, 83.3
Week 26		
n	19	15
Mean (SD)	57.55 (21.34)	56.78 (19.76)
Median	62.85	57.99

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	38.54, 73.96	47.92, 72.57
Min, Max	8.7, 92.7	7.6, 86.8
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	0.25 (12.51)	-2.60 (20.02)
Median	0.35	2.44
25th, 75th Percentile	-7.29, 5.90	-4.86, 12.10
Min, Max	-27.1, 30.9	-55.6, 15.3
Week 52		
n	18	18
Mean (SD)	55.19 (24.48)	55.47 (18.61)
Median	60.77	57.30

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.86, 75.00	49.65, 62.15
Min, Max	10.1, 88.5	1.2, 90.6
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-3.09 (13.12)	-3.83 (18.00)
Median	-2.09	-3.13
25th, 75th Percentile	-15.28, 2.09	-6.94, 7.29
Min, Max	-29.5, 17.7	-62.0, 19.5
Difference in change from baseline (95%CI)		-0.74 (-11.42, 9.93)
P-value ^b		0.8881
Hedges'g (95% CI) ^c		-0.05 (-0.70, 0.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	18	14
Mean (SD)	53.74 (22.01)	51.81 (24.31)
Median	56.43	50.52
25th, 75th Percentile	35.42, 70.14	32.29, 70.84
Min, Max	13.5, 91.0	14.6, 97.2
Week 26		
n	16	14
Mean (SD)	60.66 (17.45)	55.39 (20.71)
Median	64.24	52.95

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	48.09, 70.49	42.71, 66.46
Min, Max	25.4, 91.3	18.8, 94.8
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	5.69 (17.13)	3.58 (11.69)
Median	0.18	1.57
25th, 75th Percentile	-2.43, 10.59	-5.90, 15.97
Min, Max	-20.5, 57.3	-12.8, 22.2
Week 52		
n	18	14
Mean (SD)	59.85 (20.17)	55.61 (19.78)
Median	63.25	57.47

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.18, 73.96	43.75, 69.10
Min, Max	25.4, 89.2	8.3, 85.4
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	6.10 (17.19)	3.79 (16.97)
Median	1.91	-1.74
25th, 75th Percentile	-1.73, 8.09	-8.34, 15.28
Min, Max	-26.4, 50.7	-22.9, 34.4
Difference in change from baseline (95%CI)		-2.31 (-14.75, 10.13)
P-value ^b		0.7076
Hedges'g (95% CI) ^c		-0.13 (-0.83, 0.57)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	24	27
Mean (SD)	54.70 (21.34)	57.78 (15.61)
Median	58.86	54.17
25th, 75th Percentile	39.07, 69.97	44.10, 68.40
Min, Max	18.8, 90.6	36.5, 91.3
Week 26		
n	24	26
Mean (SD)	54.09 (18.63)	62.28 (15.35)
Median	54.52	63.89

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	39.93, 69.97	52.78, 69.79
Min, Max	21.2, 85.1	27.4, 88.9
Change from baseline to Week 26 ^a		
n	24	26
Mean (SD)	-0.61 (15.75)	4.10 (12.92)
Median	-1.22	1.52
25th, 75th Percentile	-6.25, 6.07	-3.81, 12.15
Min, Max	-42.3, 27.0	-15.6, 41.3
Week 52		
n	24	25
Mean (SD)	59.56 (17.45)	59.05 (15.29)
Median	62.12	61.81

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	44.80, 75.87	46.53, 72.23
Min, Max	28.8, 86.1	27.1, 80.6
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	4.86 (13.83)	0.01 (10.43)
Median	8.19	-1.04
25th, 75th Percentile	-3.30, 13.72	-4.17, 4.51
Min, Max	-20.1, 35.4	-23.3, 24.0
Difference in change from baseline (95%CI)		-4.85 (-11.87, 2.18)
P-value ^b		0.1716
Hedges'g (95% CI) ^c		-0.39 (-0.95, 0.18)
P-value for interaction term, treatment * [Baseline AGV]		0.8121

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	41	45
Mean (SD)	54.33 (22.23)	57.46 (17.37)
Median	57.99	57.64
25th, 75th Percentile	39.58, 69.10	47.57, 67.36
Min, Max	13.5, 92.4	14.6, 97.2
Week 26		
n	40	42
Mean (SD)	57.34 (19.89)	59.28 (16.23)
Median	63.72	58.51

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.80, 70.32	49.31, 67.02
Min, Max	8.7, 92.7	18.8, 94.8
Change from baseline to Week 26 ^a		
n	40	42
Mean (SD)	2.50 (16.97)	1.53 (12.99)
Median	1.22	1.52
25th, 75th Percentile	-5.04, 11.81	-5.90, 9.72
Min, Max	-42.3, 57.3	-36.1, 41.3
Week 52		
n	40	43
Mean (SD)	58.53 (22.05)	58.36 (12.26)
Median	61.29	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.58, 76.57	48.96, 65.28
Min, Max	10.1, 89.2	29.5, 85.4
Change from baseline to Week 52 ^a		
n	40	43
Mean (SD)	3.83 (16.75)	0.64 (12.92)
Median	1.91	-2.09
25th, 75th Percentile	-3.82, 14.24	-6.94, 10.41
Min, Max	-29.5, 50.7	-22.9, 34.4
Difference in change from baseline (95%CI)		-3.19 (-9.70, 3.32)
P-value ^b		0.3322
Hedges'g (95% CI) ^c		-0.21 (-0.64, 0.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Total Score		
Baseline		
n	20	15
Mean (SD)	57.07 (20.55)	55.61 (17.98)
Median	62.33	52.43
25th, 75th Percentile	40.11, 70.69	42.71, 72.92
Min, Max	21.5, 90.6	18.1, 83.3
Week 26		
n	19	13
Mean (SD)	56.23 (17.84)	58.20 (23.56)
Median	56.95	66.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	38.54, 73.96	41.32, 70.14
Min, Max	25.4, 85.1	7.6, 88.9
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	-0.99 (10.43)	4.10 (20.50)
Median	-3.12	5.90
25th, 75th Percentile	-7.69, 2.78	-2.43, 15.54
Min, Max	-17.0, 27.0	-55.6, 27.1
Week 52		
n	20	14
Mean (SD)	57.96 (16.87)	53.11 (27.95)
Median	62.04	64.65

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.1.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.37, 71.18	29.17, 74.31
Min, Max	25.4, 82.3	1.2, 90.6
Change from baseline to Week 52 ^a		
n	20	14
Mean (SD)	0.88 (10.80)	-3.07 (19.95)
Median	-0.20	1.36
25th, 75th Percentile	-3.13, 9.05	-9.37, 7.29
Min, Max	-20.1, 18.1	-62.0, 18.1
Difference in change from baseline (95%CI)		-3.96 (-16.24, 8.32)
P-value ^b		0.5073
Hedges'g (95% CI) ^c		-0.25 (-0.94, 0.43)
P-value for interaction term, treatment * [Ethnicity]		0.9011

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.1.2.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	33	31
Mean (SD)	50.51 (20.19)	49.33 (20.70)
Median	54.17	50.00
25th, 75th Percentile	37.50, 62.50	37.50, 62.50
Min, Max	12.5, 95.8	0.0, 91.7
Week 26		
n	31	28
Mean (SD)	49.19 (18.36)	46.67 (21.48)
Median	50.00	45.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 62.50	36.67, 62.50
Min, Max	16.7, 87.5	0.0, 83.3
Change from baseline to Week 26 ^a		
n	31	28
Mean (SD)	-2.42 (15.58)	-1.40 (19.86)
Median	0.00	0.00
25th, 75th Percentile	-8.34, 8.33	-11.25, 12.50
Min, Max	-37.5, 29.2	-66.7, 29.2
Week 52		
n	32	30
Mean (SD)	51.64 (20.78)	47.31 (20.90)
Median	56.67	47.92
25th, 75th Percentile	41.67, 64.59	37.50, 62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.002.001_qs_sum_ovr_qol_care_phy_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.2.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	8.3, 87.5	0.0, 87.5
Change from baseline to Week 52 ^a		
n	32	30
Mean (SD)	0.73 (16.67)	-1.44 (21.07)
Median	0.00	0.00
25th, 75th Percentile	-10.42, 11.25	-12.50, 12.50
Min, Max	-29.2, 37.5	-70.8, 37.5
Difference in change from baseline (95%CI)		-2.17 (-11.79, 7.45)
P-value ^b		0.6530
Hedges'g (95% CI) ^c		-0.11 (-0.61, 0.39)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.2.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	28	29
Mean (SD)	43.16 (25.94)	47.10 (18.10)
Median	41.67	45.00
25th, 75th Percentile	20.83, 60.42	37.50, 58.33
Min, Max	0.0, 87.5	20.8, 91.7
Week 26		
n	28	27
Mean (SD)	44.82 (25.26)	51.73 (19.53)
Median	54.17	45.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.2.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	22.92, 62.50	37.50, 66.67
Min, Max	4.2, 83.3	16.7, 87.5
Change from baseline to Week 26 ^a		
n	28	27
Mean (SD)	1.67 (20.25)	3.92 (15.67)
Median	4.16	4.17
25th, 75th Percentile	-8.33, 14.58	-8.33, 12.50
Min, Max	-54.2, 50.0	-33.3, 45.8
Week 52		
n	28	27
Mean (SD)	49.85 (22.47)	49.07 (20.06)
Median	54.17	54.17
25th, 75th Percentile	27.09, 66.67	33.33, 62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 83.3	12.5, 87.5
Change from baseline to Week 52 ^a		
n	28	27
Mean (SD)	6.70 (15.85)	1.27 (14.24)
Median	6.25	-4.17
25th, 75th Percentile	-6.25, 16.66	-8.33, 12.50
Min, Max	-20.8, 45.8	-25.0, 29.2
Difference in change from baseline (95%CI)		-5.43 (-13.59, 2.73)
P-value ^b		0.1876
Hedges'g (95% CI) ^c		-0.35 (-0.89, 0.18)
P-value for interaction term, treatment * [Sex]		0.6110

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	24	31
Mean (SD)	44.27 (21.97)	47.96 (19.28)
Median	39.59	45.83
25th, 75th Percentile	29.17, 58.34	37.50, 58.33
Min, Max	4.2, 83.3	0.0, 91.7
Week 26		
n	24	28
Mean (SD)	39.76 (21.46)	46.16 (21.50)
Median	33.34	45.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	22.92, 58.33	33.33, 60.42
Min, Max	8.3, 83.3	0.0, 80.0
Change from baseline to Week 26 ^a		
n	24	28
Mean (SD)	-4.52 (18.26)	-1.73 (17.67)
Median	0.00	0.00
25th, 75th Percentile	-16.67, 8.33	-8.34, 6.25
Min, Max	-54.2, 20.8	-66.7, 35.0
Week 52		
n	23	31
Mean (SD)	48.41 (24.23)	44.89 (21.48)
Median	55.00	50.00
25th, 75th Percentile	25.00, 66.67	25.00, 58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	8.3, 83.3	0.0, 87.5
Change from baseline to Week 52 ^a		
n	23	31
Mean (SD)	3.84 (16.71)	-3.06 (21.28)
Median	0.00	-4.17
25th, 75th Percentile	-8.34, 16.66	-12.50, 12.50
Min, Max	-29.2, 37.5	-70.8, 37.5
Difference in change from baseline (95%CI)		-6.90 (-17.66, 3.85)
P-value ^b		0.2034
Hedges'g (95% CI) ^c		-0.35 (-0.89, 0.20)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	24	17
Mean (SD)	50.00 (25.09)	44.36 (18.51)
Median	54.17	41.67
25th, 75th Percentile	37.50, 64.59	37.50, 50.00
Min, Max	0.0, 95.8	16.7, 79.2
Week 26		
n	23	16
Mean (SD)	52.03 (21.97)	46.09 (19.51)
Median	55.00	43.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	41.67, 66.67	33.33, 60.42
Min, Max	4.2, 83.3	16.7, 87.5
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	2.03 (16.69)	1.82 (18.25)
Median	0.00	2.08
25th, 75th Percentile	-8.33, 12.50	-8.33, 16.67
Min, Max	-37.5, 34.2	-33.3, 29.2
Week 52		
n	24	16
Mean (SD)	52.15 (20.18)	49.90 (19.45)
Median	52.09	41.67
25th, 75th Percentile	41.67, 66.67	35.42, 65.84

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 83.3	12.5, 79.2
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	2.15 (17.14)	5.63 (14.44)
Median	2.08	2.09
25th, 75th Percentile	-12.50, 12.50	-4.17, 18.75
Min, Max	-29.2, 37.5	-20.8, 29.2
Difference in change from baseline (95%CI)		3.47 (-7.07, 14.01)
P-value ^b		0.5089
Hedges'g (95% CI) ^c		0.21 (-0.42, 0.84)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	13	12
Mean (SD)	47.12 (22.46)	54.52 (20.75)
Median	54.17	56.25
25th, 75th Percentile	37.50, 62.50	33.34, 64.59
Min, Max	12.5, 83.3	29.2, 91.7
Week 26		
n	12	11
Mean (SD)	52.43 (19.58)	61.21 (15.93)
Median	54.17	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	47.92, 62.50	45.83, 75.00
Min, Max	8.3, 87.5	40.0, 87.5
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	2.78 (19.41)	7.80 (18.07)
Median	-2.09	8.33
25th, 75th Percentile	-8.33, 10.42	-4.17, 16.67
Min, Max	-20.8, 50.0	-16.7, 45.8
Week 52		
n	13	10
Mean (SD)	52.56 (19.51)	55.42 (17.46)
Median	58.33	54.17
25th, 75th Percentile	45.83, 62.50	41.67, 70.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 87.5	33.3, 87.5
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	5.45 (15.63)	-0.42 (9.10)
Median	4.17	0.00
25th, 75th Percentile	-4.17, 8.33	-8.33, 8.33
Min, Max	-20.8, 45.8	-12.5, 12.5
Difference in change from baseline (95%CI)		-5.86 (-17.44, 5.71)
P-value ^b		0.3038
Hedges'g (95% CI) ^c		-0.43 (-1.26, 0.41)
P-value for interaction term, treatment * [Age at Baseline]		0.3426

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	48	48
Mean (SD)	45.75 (23.62)	46.08 (18.32)
Median	50.00	45.83
25th, 75th Percentile	29.17, 60.42	37.50, 56.25
Min, Max	0.0, 95.8	0.0, 91.7
Week 26		
n	46	44
Mean (SD)	45.92 (21.86)	45.72 (20.30)
Median	50.00	43.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 62.50	33.33, 60.42
Min, Max	4.2, 83.3	0.0, 83.3
Change from baseline to Week 26 ^a		
n	46	44
Mean (SD)	-0.36 (18.96)	0.28 (18.32)
Median	0.00	0.00
25th, 75th Percentile	-8.34, 12.50	-8.34, 14.58
Min, Max	-54.2, 50.0	-66.7, 35.0
Week 52		
n	47	47
Mean (SD)	50.32 (21.55)	45.27 (19.77)
Median	54.17	45.83
25th, 75th Percentile	25.00, 66.67	33.33, 62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	8.3, 83.3	0.0, 87.5
Change from baseline to Week 52 ^a		
n	47	47
Mean (SD)	4.40 (17.51)	-0.37 (19.46)
Median	4.16	0.00
25th, 75th Percentile	-8.34, 16.66	-12.50, 12.50
Min, Max	-29.2, 45.8	-70.8, 37.5
Difference in change from baseline (95%CI)		-4.77 (-12.35, 2.82)
P-value ^b		0.2149
Hedges'g (95% CI) ^c		-0.26 (-0.66, 0.15)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.002.003_qs_sum_ovr_qol_care_phy_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.2.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	13	12
Mean (SD)	52.24 (21.15)	56.95 (21.71)
Median	58.33	54.17
25th, 75th Percentile	37.50, 62.50	39.59, 77.09
Min, Max	12.5, 83.3	29.2, 91.7
Week 26		
n	13	11
Mean (SD)	51.35 (22.02)	62.88 (15.53)
Median	54.17	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.83, 58.33	50.00, 75.00
Min, Max	4.2, 87.5	41.7, 87.5
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	-0.90 (14.21)	4.92 (16.75)
Median	-8.33	0.00
25th, 75th Percentile	-8.33, 4.16	-4.17, 12.50
Min, Max	-20.8, 34.2	-16.7, 45.8
Week 52		
n	13	10
Mean (SD)	52.57 (21.69)	61.67 (18.19)
Median	58.33	66.67
25th, 75th Percentile	41.67, 66.67	41.67, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 87.5	33.3, 87.5
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	0.32 (11.72)	0.83 (9.58)
Median	4.17	0.00
25th, 75th Percentile	-8.33, 4.17	-4.17, 4.17
Min, Max	-20.8, 20.8	-12.5, 20.8
Difference in change from baseline (95%CI)		0.51 (-8.98, 10.01)
P-value ^b		0.9119
Hedges'g (95% CI) ^c		0.05 (-0.78, 0.87)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5172

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	10	15
Mean (SD)	37.50 (27.99)	42.50 (13.20)
Median	37.50	41.67
25th, 75th Percentile	8.33, 62.50	29.17, 50.00
Min, Max	0.0, 79.2	20.8, 66.7
Week 26		
n	10	12
Mean (SD)	32.50 (26.34)	47.08 (11.78)
Median	25.00	45.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	12.50, 58.33	37.50, 54.17
Min, Max	4.2, 83.3	33.3, 70.8
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-5.00 (21.32)	5.76 (13.04)
Median	2.08	6.25
25th, 75th Percentile	-8.33, 4.17	-6.26, 16.67
Min, Max	-54.2, 16.7	-12.5, 29.2
Week 52		
n	10	13
Mean (SD)	44.58 (28.87)	44.74 (15.97)
Median	39.59	41.67
25th, 75th Percentile	20.83, 75.00	37.50, 58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	8.3, 83.3	16.7, 66.7
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	7.08 (13.33)	4.36 (15.36)
Median	12.50	4.16
25th, 75th Percentile	0.00, 16.66	-8.33, 20.83
Min, Max	-25.0, 20.8	-12.5, 29.2
Difference in change from baseline (95%CI)		-2.72 (-15.43, 9.98)
P-value ^b		0.6605
Hedges'g (95% CI) ^c		-0.18 (-1.00, 0.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	24	18
Mean (SD)	48.61 (23.01)	45.60 (17.00)
Median	50.00	45.83
25th, 75th Percentile	37.50, 62.50	29.17, 54.17
Min, Max	12.5, 95.8	20.8, 75.0
Week 26		
n	22	18
Mean (SD)	49.81 (18.96)	36.81 (18.65)
Median	54.17	37.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.17, 62.50	25.00, 45.83
Min, Max	16.7, 87.5	4.2, 66.7
Change from baseline to Week 26 ^a		
n	22	18
Mean (SD)	-0.19 (19.31)	-8.80 (19.90)
Median	0.00	-8.33
25th, 75th Percentile	-8.34, 8.33	-20.83, 4.17
Min, Max	-37.5, 50.0	-66.7, 16.7
Week 52		
n	23	18
Mean (SD)	53.19 (19.54)	37.04 (21.34)
Median	58.33	33.33
25th, 75th Percentile	45.83, 66.67	25.00, 50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	8.3, 87.5	0.0, 79.2
Change from baseline to Week 52 ^a		
n	23	18
Mean (SD)	4.09 (16.67)	-8.57 (23.15)
Median	4.17	-2.09
25th, 75th Percentile	-8.33, 12.50	-20.83, 4.16
Min, Max	-29.2, 45.8	-70.8, 33.3
Difference in change from baseline (95%CI)		-12.66 (-25.24, -0.08)
P-value ^b		0.0486
Hedges'g (95% CI) ^c		-0.63 (-1.26, 0.01)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	19	22
Mean (SD)	47.81 (21.49)	50.72 (23.28)
Median	54.17	50.00
25th, 75th Percentile	33.33, 58.33	37.50, 66.67
Min, Max	12.5, 87.5	0.0, 91.7
Week 26		
n	19	20
Mean (SD)	46.97 (22.66)	56.71 (20.92)
Median	54.17	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.17, 62.50	43.75, 75.00
Min, Max	4.2, 83.3	0.0, 87.5
Change from baseline to Week 26 ^a		
n	19	20
Mean (SD)	-0.83 (16.56)	6.75 (16.45)
Median	-4.17	2.09
25th, 75th Percentile	-8.34, 12.50	-4.17, 18.75
Min, Max	-33.3, 34.2	-16.7, 45.8
Week 52		
n	19	21
Mean (SD)	48.25 (23.05)	56.15 (18.89)
Median	45.83	58.33
25th, 75th Percentile	25.00, 66.67	41.67, 70.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 83.3	12.5, 87.5
Change from baseline to Week 52 ^a		
n	19	21
Mean (SD)	0.44 (17.83)	4.40 (13.13)
Median	0.00	4.17
25th, 75th Percentile	-12.50, 12.50	-4.17, 12.50
Min, Max	-29.2, 37.5	-16.7, 37.5
Difference in change from baseline (95%CI)		3.97 (-5.99, 13.92)
P-value ^b		0.4252
Hedges'g (95% CI) ^c		0.25 (-0.37, 0.87)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	8	5
Mean (SD)	53.13 (21.45)	64.17 (18.30)
Median	56.25	62.50
25th, 75th Percentile	41.67, 68.75	58.33, 79.17
Min, Max	12.5, 79.2	37.5, 83.3
Week 26		
n	8	5
Mean (SD)	58.33 (13.55)	68.33 (17.33)
Median	54.17	66.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 66.67	58.33, 83.33
Min, Max	41.7, 83.3	45.8, 87.5
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	5.21 (13.50)	4.17 (15.59)
Median	2.08	8.33
25th, 75th Percentile	-2.09, 12.50	-4.17, 8.33
Min, Max	-12.5, 29.2	-16.7, 25.0
Week 52		
n	8	5
Mean (SD)	57.81 (9.03)	63.33 (9.50)
Median	58.34	62.50
25th, 75th Percentile	50.00, 64.59	54.17, 70.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	45.8, 70.8	54.2, 75.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	4.69 (17.60)	-0.83 (14.85)
Median	0.00	-4.17
25th, 75th Percentile	-6.25, 12.50	-8.33, -4.16
Min, Max	-12.5, 37.5	-12.5, 25.0
Difference in change from baseline (95%CI)		-5.52 (-26.41, 15.37)
P-value ^b		0.5727
Hedges'g (95% CI) ^c		-0.31 (-1.43, 0.82)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.1932

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	19	19
Mean (SD)	48.90 (24.33)	50.22 (17.97)
Median	54.17	54.17
25th, 75th Percentile	33.33, 66.67	29.17, 66.67
Min, Max	8.3, 95.8	20.8, 75.0
Week 26		
n	19	15
Mean (SD)	48.46 (22.06)	42.50 (23.21)
Median	54.17	41.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 62.50	25.00, 62.50
Min, Max	4.2, 87.5	4.2, 83.3
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	-0.44 (14.63)	-6.94 (23.18)
Median	0.00	-8.33
25th, 75th Percentile	-8.33, 12.50	-20.83, 8.33
Min, Max	-37.5, 20.8	-66.7, 25.0
Week 52		
n	18	18
Mean (SD)	48.38 (25.73)	45.37 (21.38)
Median	54.17	41.67
25th, 75th Percentile	25.00, 66.67	33.33, 58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 87.5	0.0, 87.5
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-1.16 (15.51)	-3.94 (22.12)
Median	2.09	-2.08
25th, 75th Percentile	-12.50, 12.50	-16.67, 12.50
Min, Max	-29.2, 25.0	-70.8, 33.3
Difference in change from baseline (95%CI)		-2.78 (-15.72, 10.16)
P-value ^b		0.6654
Hedges'g (95% CI) ^c		-0.14 (-0.80, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	18	14
Mean (SD)	49.08 (22.03)	43.16 (24.82)
Median	52.09	37.50
25th, 75th Percentile	37.50, 62.50	25.00, 58.33
Min, Max	12.5, 79.2	0.0, 91.7
Week 26		
n	16	14
Mean (SD)	51.30 (20.40)	45.42 (23.85)
Median	52.09	41.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	33.34, 64.59	37.50, 58.33
Min, Max	16.7, 83.3	0.0, 87.5
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	0.26 (19.21)	2.26 (13.85)
Median	0.00	0.00
25th, 75th Percentile	-8.33, 4.16	-8.34, 16.67
Min, Max	-33.3, 50.0	-20.8, 29.2
Week 52		
n	18	14
Mean (SD)	51.48 (20.84)	46.73 (22.18)
Median	58.33	43.75
25th, 75th Percentile	41.67, 62.50	37.50, 62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 83.3	12.5, 87.5
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	2.41 (16.66)	3.57 (18.34)
Median	0.00	-2.09
25th, 75th Percentile	-4.17, 4.17	-12.50, 20.83
Min, Max	-29.2, 45.8	-25.0, 37.5
Difference in change from baseline (95%CI)		1.16 (-11.50, 13.83)
P-value ^b		0.8523
Hedges'g (95% CI) ^c		0.07 (-0.63, 0.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	24	27
Mean (SD)	44.27 (23.60)	49.51 (17.32)
Median	50.00	45.83
25th, 75th Percentile	27.08, 56.25	37.50, 62.50
Min, Max	0.0, 87.5	25.0, 91.7
Week 26		
n	24	26
Mean (SD)	43.26 (22.80)	55.00 (15.55)
Median	50.00	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	22.92, 60.42	41.67, 66.67
Min, Max	4.2, 83.3	29.2, 80.0
Change from baseline to Week 26 ^a		
n	24	26
Mean (SD)	-1.01 (20.00)	5.35 (15.42)
Median	2.08	2.09
25th, 75th Percentile	-12.50, 10.42	-4.17, 12.50
Min, Max	-54.2, 34.2	-16.7, 45.8
Week 52		
n	24	25
Mean (SD)	52.12 (18.96)	50.93 (18.98)
Median	54.59	54.17
25th, 75th Percentile	41.67, 66.67	37.50, 65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	20.8, 83.3	12.5, 79.2
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	7.85 (16.49)	0.47 (14.59)
Median	8.33	0.00
25th, 75th Percentile	0.00, 20.83	-8.33, 8.33
Min, Max	-29.2, 37.5	-41.7, 25.0
Difference in change from baseline (95%CI)		-7.38 (-16.32, 1.56)
P-value ^b		0.1034
Hedges'g (95% CI) ^c		-0.47 (-1.03, 0.10)
P-value for interaction term, treatment * [Baseline AGV]		0.5464

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	41	45
Mean (SD)	45.12 (24.58)	47.96 (20.05)
Median	45.83	45.83
25th, 75th Percentile	33.33, 58.33	37.50, 58.33
Min, Max	0.0, 95.8	0.0, 91.7
Week 26		
n	40	42
Mean (SD)	47.50 (22.30)	47.88 (19.84)
Median	52.09	45.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.09, 62.50	37.50, 62.50
Min, Max	4.2, 87.5	0.0, 87.5
Change from baseline to Week 26 ^a		
n	40	42
Mean (SD)	1.67 (19.33)	-0.24 (14.83)
Median	4.16	0.00
25th, 75th Percentile	-8.33, 14.58	-8.34, 8.33
Min, Max	-54.2, 50.0	-33.3, 45.8
Week 52		
n	40	43
Mean (SD)	50.10 (22.46)	48.45 (17.35)
Median	52.09	50.00
25th, 75th Percentile	41.67, 66.67	37.50, 58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 87.5	12.5, 87.5
Change from baseline to Week 52 ^a		
n	40	43
Mean (SD)	4.79 (18.33)	0.48 (14.30)
Median	4.17	0.00
25th, 75th Percentile	-10.42, 16.66	-12.50, 12.50
Min, Max	-29.2, 45.8	-25.0, 37.5
Difference in change from baseline (95%CI)		-4.31 (-11.46, 2.85)
P-value ^b		0.2345
Hedges'g (95% CI) ^c		-0.26 (-0.69, 0.17)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Physical Score		
Baseline		
n	20	15
Mean (SD)	51.25 (19.69)	49.11 (17.70)
Median	54.17	45.83
25th, 75th Percentile	37.50, 62.50	37.50, 62.50
Min, Max	16.7, 87.5	16.7, 79.2
Week 26		
n	19	13
Mean (SD)	46.32 (21.35)	53.27 (22.91)
Median	50.00	54.17

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 62.50	37.50, 70.83
Min, Max	16.7, 83.3	4.2, 87.5
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	-5.00 (13.85)	5.90 (25.87)
Median	-4.17	8.33
25th, 75th Percentile	-8.34, 0.00	-4.17, 16.67
Min, Max	-33.3, 34.2	-66.7, 35.0
Week 52		
n	20	14
Mean (SD)	52.21 (19.65)	47.20 (28.44)
Median	57.50	62.50
25th, 75th Percentile	35.42, 66.67	20.83, 66.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.2.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	16.7, 79.2	0.0, 87.5
Change from baseline to Week 52 ^a		
n	20	14
Mean (SD)	0.96 (11.75)	-2.14 (27.17)
Median	0.00	0.00
25th, 75th Percentile	-4.17, 7.50	-12.50, 20.83
Min, Max	-29.2, 20.8	-70.8, 29.2
Difference in change from baseline (95%CI)		-3.10 (-19.44, 13.23)
P-value ^b		0.6930
Hedges'g (95% CI) ^c		-0.16 (-0.84, 0.53)
P-value for interaction term, treatment * [Ethnicity]		0.8664

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	33	31
Mean (SD)	61.05 (21.32)	56.63 (21.56)
Median	62.50	59.38
25th, 75th Percentile	46.88, 75.00	37.50, 75.00
Min, Max	21.9, 93.8	0.0, 93.8
Week 26		
n	31	28
Mean (SD)	60.49 (19.17)	55.09 (23.24)
Median	59.38	54.69

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.003.001_qs_sum_ovr_qol_care_soc_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.3.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 75.00	42.19, 76.57
Min, Max	21.9, 93.8	0.0, 89.3
Change from baseline to Week 26 ^a		
n	31	28
Mean (SD)	-1.58 (15.48)	-0.02 (16.99)
Median	0.00	3.12
25th, 75th Percentile	-12.50, 9.37	-9.38, 9.83
Min, Max	-34.4, 34.4	-53.1, 34.4
Week 52		
n	32	30
Mean (SD)	62.56 (22.97)	54.69 (21.83)
Median	67.19	59.38
25th, 75th Percentile	48.44, 79.69	43.75, 71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 93.8	0.0, 87.5
Change from baseline to Week 52 ^a		
n	32	30
Mean (SD)	0.96 (17.93)	-1.85 (19.97)
Median	-1.56	-3.13
25th, 75th Percentile	-12.50, 17.19	-9.38, 9.37
Min, Max	-28.1, 40.6	-53.1, 46.9
Difference in change from baseline (95%CI)		-2.81 (-12.44, 6.82)
P-value ^b		0.5618
Hedges'g (95% CI) ^c		-0.15 (-0.64, 0.35)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.3.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	28	29
Mean (SD)	53.05 (22.20)	58.19 (19.63)
Median	46.88	56.25
25th, 75th Percentile	35.94, 75.00	46.88, 71.88
Min, Max	15.6, 93.8	18.8, 100.0
Week 26		
n	28	27
Mean (SD)	57.37 (19.51)	65.71 (16.94)
Median	64.07	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.3.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.76, 68.75	50.00, 78.13
Min, Max	18.8, 90.6	34.4, 96.9
Change from baseline to Week 26 ^a		
n	28	27
Mean (SD)	4.32 (16.43)	6.56 (17.84)
Median	1.56	6.25
25th, 75th Percentile	-6.25, 12.50	0.00, 18.75
Min, Max	-29.0, 50.0	-53.1, 37.5
Week 52		
n	28	27
Mean (SD)	59.49 (20.05)	61.13 (15.31)
Median	57.82	62.50
25th, 75th Percentile	46.88, 78.13	53.13, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.8, 90.6	21.9, 81.3
Change from baseline to Week 52 ^a		
n	28	27
Mean (SD)	6.44 (14.65)	1.87 (16.81)
Median	6.25	0.00
25th, 75th Percentile	-1.57, 12.50	-9.37, 9.37
Min, Max	-18.8, 43.8	-37.5, 53.1
Difference in change from baseline (95%CI)		-4.57 (-13.09, 3.95)
P-value ^b		0.2866
Hedges'g (95% CI) ^c		-0.29 (-0.82, 0.25)
P-value for interaction term, treatment * [Sex]		0.7862

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	24	31
Mean (SD)	57.85 (21.08)	56.86 (20.59)
Median	57.82	56.25
25th, 75th Percentile	40.63, 73.44	37.50, 71.88
Min, Max	21.9, 93.8	0.0, 90.6
Week 26		
n	24	28
Mean (SD)	54.82 (20.95)	60.30 (21.57)
Median	53.13	57.82

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.94, 70.32	53.13, 75.01
Min, Max	21.9, 90.6	0.0, 93.8
Change from baseline to Week 26 ^a		
n	24	28
Mean (SD)	-3.03 (15.14)	4.16 (16.34)
Median	-3.13	5.14
25th, 75th Percentile	-12.50, 4.69	0.00, 15.63
Min, Max	-31.3, 34.4	-53.1, 34.4
Week 52		
n	23	31
Mean (SD)	65.36 (23.33)	57.48 (19.38)
Median	75.00	59.38
25th, 75th Percentile	40.63, 87.50	46.88, 71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 93.8	0.0, 87.5
Change from baseline to Week 52 ^a		
n	23	31
Mean (SD)	6.89 (16.88)	0.62 (19.27)
Median	6.25	0.00
25th, 75th Percentile	-6.25, 21.87	-9.37, 12.50
Min, Max	-28.1, 43.8	-53.1, 46.9
Difference in change from baseline (95%CI)		-6.27 (-16.37, 3.83)
P-value ^b		0.2186
Hedges'g (95% CI) ^c		-0.34 (-0.88, 0.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	24	17
Mean (SD)	57.68 (22.93)	53.86 (19.38)
Median	59.38	53.13
25th, 75th Percentile	39.07, 75.00	37.50, 71.88
Min, Max	15.6, 93.8	18.8, 90.6
Week 26		
n	23	16
Mean (SD)	59.65 (18.44)	53.52 (19.60)
Median	65.63	48.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 75.00	39.07, 71.88
Min, Max	18.8, 87.5	18.8, 84.4
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	2.17 (16.41)	-0.39 (21.71)
Median	3.12	3.12
25th, 75th Percentile	-6.25, 9.38	-12.50, 9.38
Min, Max	-34.4, 34.4	-53.1, 34.4
Week 52		
n	24	16
Mean (SD)	55.29 (20.40)	55.28 (19.72)
Median	56.26	57.82
25th, 75th Percentile	43.76, 71.88	46.88, 71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.6, 90.6	3.1, 78.1
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	-2.40 (16.06)	1.37 (21.71)
Median	-1.56	0.00
25th, 75th Percentile	-15.62, 6.25	-6.25, 9.38
Min, Max	-25.0, 40.6	-37.5, 53.1
Difference in change from baseline (95%CI)		3.77 (-8.32, 15.85)
P-value ^b		0.5317
Hedges'g (95% CI) ^c		0.20 (-0.44, 0.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	13	12
Mean (SD)	55.94 (23.22)	63.73 (22.02)
Median	53.13	68.31
25th, 75th Percentile	31.25, 75.00	43.76, 75.00
Min, Max	25.0, 93.8	31.3, 100.0
Week 26		
n	12	11
Mean (SD)	66.15 (16.04)	70.17 (18.61)
Median	67.19	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 73.44	65.63, 78.13
Min, Max	40.6, 93.8	28.1, 96.9
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	7.89 (15.99)	6.05 (14.37)
Median	6.25	3.13
25th, 75th Percentile	-3.12, 12.50	-3.12, 12.50
Min, Max	-9.4, 50.0	-15.6, 37.5
Week 52		
n	13	10
Mean (SD)	64.43 (19.20)	62.50 (18.52)
Median	65.63	67.19
25th, 75th Percentile	53.13, 81.25	53.13, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.8, 90.6	25.0, 81.3
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	8.48 (14.70)	-4.60 (8.43)
Median	6.25	-3.13
25th, 75th Percentile	0.89, 18.75	-6.25, 0.89
Min, Max	-12.5, 40.6	-18.8, 6.3
Difference in change from baseline (95%CI)		-13.08 (-23.93, -2.23)
P-value ^b		0.0205
Hedges'g (95% CI) ^c		-1.02 (-1.89, -0.13)
P-value for interaction term, treatment * [Age at Baseline]		0.1645

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	48	48
Mean (SD)	57.64 (21.55)	55.26 (20.26)
Median	59.38	56.25
25th, 75th Percentile	39.07, 75.00	37.50, 69.87
Min, Max	21.9, 93.8	0.0, 90.6
Week 26		
n	46	44
Mean (SD)	57.82 (18.99)	58.40 (21.84)
Median	62.51	57.82

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.3.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 71.88	45.32, 78.13
Min, Max	21.9, 90.6	0.0, 93.8
Change from baseline to Week 26 ^a		
n	46	44
Mean (SD)	-0.36 (16.90)	3.81 (17.73)
Median	0.00	5.14
25th, 75th Percentile	-12.50, 9.38	0.00, 14.06
Min, Max	-34.4, 50.0	-53.1, 34.4
Week 52		
n	47	47
Mean (SD)	61.54 (22.70)	55.93 (19.54)
Median	65.63	59.38
25th, 75th Percentile	40.63, 81.25	46.88, 68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 93.8	0.0, 87.5
Change from baseline to Week 52 ^a		
n	47	47
Mean (SD)	3.61 (17.67)	0.76 (19.35)
Median	3.13	0.00
25th, 75th Percentile	-9.38, 15.63	-9.37, 9.38
Min, Max	-28.1, 43.8	-53.1, 53.1
Difference in change from baseline (95%CI)		-2.85 (-10.44, 4.74)
P-value ^b		0.4579
Hedges'g (95% CI) ^c		-0.15 (-0.56, 0.25)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.3.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	13	12
Mean (SD)	56.42 (24.08)	65.89 (19.96)
Median	53.13	70.32
25th, 75th Percentile	40.63, 75.00	50.01, 75.00
Min, Max	15.6, 93.8	34.4, 100.0
Week 26		
n	13	11
Mean (SD)	63.22 (20.26)	67.90 (15.12)
Median	62.50	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.3.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 71.88	50.00, 75.00
Min, Max	18.8, 93.8	46.9, 96.9
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	6.80 (11.65)	0.85 (17.51)
Median	6.25	0.00
25th, 75th Percentile	-3.12, 9.38	-9.38, 6.25
Min, Max	-8.5, 34.4	-28.1, 37.5
Week 52		
n	13	10
Mean (SD)	59.62 (17.33)	66.25 (15.15)
Median	59.38	73.44
25th, 75th Percentile	50.00, 68.75	53.13, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	21.9, 90.6	37.5, 81.3
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	3.19 (12.42)	-4.06 (13.66)
Median	6.25	-1.56
25th, 75th Percentile	-9.37, 12.50	-18.75, 6.25
Min, Max	-15.6, 18.8	-28.1, 15.6
Difference in change from baseline (95%CI)		-7.26 (-18.60, 4.09)
P-value ^b		0.1977
Hedges'g (95% CI) ^c		-0.54 (-1.37, 0.31)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5947

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	10	15
Mean (SD)	55.40 (24.05)	50.21 (14.20)
Median	50.01	53.13
25th, 75th Percentile	37.50, 82.14	37.50, 59.38
Min, Max	25.0, 93.8	18.8, 71.9
Week 26		
n	10	12
Mean (SD)	47.50 (23.10)	67.45 (14.44)
Median	35.94	70.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	28.13, 68.75	57.82, 78.13
Min, Max	25.0, 90.6	40.6, 84.4
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-7.90 (11.70)	17.19 (12.54)
Median	-9.38	15.63
25th, 75th Percentile	-12.50, -3.12	9.38, 26.57
Min, Max	-29.0, 15.6	-9.4, 34.4
Week 52		
n	10	13
Mean (SD)	57.19 (24.12)	58.42 (12.39)
Median	54.69	62.50
25th, 75th Percentile	37.50, 78.13	53.13, 65.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.8, 90.6	34.4, 75.0
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	1.79 (13.47)	9.13 (17.33)
Median	2.68	6.25
25th, 75th Percentile	-9.37, 12.50	-3.12, 15.62
Min, Max	-18.8, 18.8	-9.4, 53.1
Difference in change from baseline (95%CI)		7.35 (-6.46, 21.16)
P-value ^b		0.2810
Hedges'g (95% CI) ^c		0.45 (-0.39, 1.28)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	24	18
Mean (SD)	57.13 (22.71)	56.03 (16.34)
Median	57.82	54.69
25th, 75th Percentile	35.94, 73.44	40.63, 67.86
Min, Max	21.9, 93.8	31.3, 90.6
Week 26		
n	22	18
Mean (SD)	60.66 (17.30)	51.05 (18.78)
Median	62.51	53.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 71.88	40.63, 59.38
Min, Max	21.9, 93.8	0.0, 84.4
Change from baseline to Week 26 ^a		
n	22	18
Mean (SD)	2.46 (16.73)	-4.99 (20.63)
Median	3.13	3.12
25th, 75th Percentile	-8.48, 12.50	-15.62, 9.38
Min, Max	-34.4, 50.0	-53.1, 15.6
Week 52		
n	23	18
Mean (SD)	64.35 (22.31)	48.96 (18.99)
Median	68.75	54.69
25th, 75th Percentile	50.00, 81.25	40.63, 59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 93.8	0.0, 71.9
Change from baseline to Week 52 ^a		
n	23	18
Mean (SD)	6.64 (15.86)	-7.07 (19.84)
Median	6.25	-3.13
25th, 75th Percentile	-3.12, 18.75	-15.63, 0.89
Min, Max	-25.0, 40.6	-53.1, 28.1
Difference in change from baseline (95%CI)		-13.71 (-24.98, -2.44)
P-value ^b		0.0184
Hedges'g (95% CI) ^c		-0.76 (-1.39, -0.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	19	22
Mean (SD)	54.11 (21.75)	59.80 (26.28)
Median	53.13	62.50
25th, 75th Percentile	31.25, 75.00	34.38, 84.38
Min, Max	15.6, 90.6	0.0, 100.0
Week 26		
n	19	20
Mean (SD)	58.55 (19.03)	61.77 (25.34)
Median	62.50	64.07

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 71.88	45.32, 82.82
Min, Max	18.8, 87.5	15.6, 96.9
Change from baseline to Week 26 ^a		
n	19	20
Mean (SD)	4.44 (18.64)	3.64 (14.02)
Median	3.12	3.13
25th, 75th Percentile	-6.25, 18.75	-1.56, 9.38
Min, Max	-31.3, 34.4	-28.1, 37.5
Week 52		
n	19	21
Mean (SD)	57.73 (21.18)	61.33 (21.99)
Median	56.25	68.75
25th, 75th Percentile	46.88, 75.00	46.88, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.6, 87.5	3.1, 87.5
Change from baseline to Week 52 ^a		
n	19	21
Mean (SD)	3.62 (19.55)	0.32 (17.70)
Median	0.00	-3.13
25th, 75th Percentile	-12.50, 18.75	-9.37, 9.38
Min, Max	-28.1, 43.8	-28.1, 46.9
Difference in change from baseline (95%CI)		-3.30 (-15.22, 8.62)
P-value ^b		0.5787
Hedges'g (95% CI) ^c		-0.17 (-0.79, 0.45)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	8	5
Mean (SD)	68.36 (16.99)	73.13 (13.55)
Median	71.88	71.88
25th, 75th Percentile	62.51, 75.00	68.75, 75.00
Min, Max	34.4, 93.8	56.3, 93.8
Week 26		
n	8	5
Mean (SD)	69.92 (14.65)	70.63 (9.53)
Median	73.44	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 79.69	65.63, 78.13
Min, Max	46.9, 87.5	56.3, 78.1
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	1.56 (8.99)	-2.50 (7.78)
Median	1.56	0.00
25th, 75th Percentile	-4.69, 7.82	-3.12, 3.12
Min, Max	-12.5, 15.6	-15.6, 3.1
Week 52		
n	8	5
Mean (SD)	64.85 (18.35)	72.50 (5.13)
Median	62.51	75.00
25th, 75th Percentile	50.01, 81.25	68.75, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.003.005_qs_sum_ovr_qol_care_soc_bhgt_301_fas.pdf+rtf

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Table 14.2.8.1.3.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	40.6, 90.6	65.6, 78.1
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-3.51 (14.70)	-0.62 (11.78)
Median	-7.81	0.00
25th, 75th Percentile	-14.06, 7.81	-3.12, 6.25
Min, Max	-21.9, 21.9	-18.8, 12.5
Difference in change from baseline (95%CI)		2.89 (-14.31, 20.09)
P-value ^b		0.7186
Hedges'g (95% CI) ^c		0.20 (-0.93, 1.31)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.1133

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	19	19
Mean (SD)	58.67 (23.45)	60.81 (17.11)
Median	53.13	62.50
25th, 75th Percentile	40.63, 75.00	50.00, 75.00
Min, Max	15.6, 93.8	31.3, 90.6
Week 26		
n	19	15
Mean (SD)	56.25 (21.50)	59.79 (24.86)
Median	59.38	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 75.00	43.75, 78.13
Min, Max	18.8, 90.6	0.0, 93.8
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	-2.42 (14.77)	-0.56 (24.21)
Median	-3.12	6.25
25th, 75th Percentile	-12.50, 6.25	-3.12, 10.27
Min, Max	-34.4, 31.3	-53.1, 34.4
Week 52		
n	18	18
Mean (SD)	56.08 (26.79)	55.21 (20.11)
Median	59.38	57.82
25th, 75th Percentile	37.50, 78.13	50.00, 68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 93.8	0.0, 87.5
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-3.42 (15.73)	-5.68 (17.54)
Median	-6.25	-3.13
25th, 75th Percentile	-15.62, 6.25	-9.37, 0.89
Min, Max	-25.0, 31.3	-53.1, 21.9
Difference in change from baseline (95%CI)		-2.26 (-13.54, 9.03)
P-value ^b		0.6871
Hedges'g (95% CI) ^c		-0.13 (-0.79, 0.52)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	18	14
Mean (SD)	57.29 (22.64)	50.90 (26.90)
Median	64.07	51.57
25th, 75th Percentile	31.25, 75.00	34.38, 71.88
Min, Max	25.0, 93.8	0.0, 100.0
Week 26		
n	16	14
Mean (SD)	64.85 (17.51)	56.70 (23.06)
Median	68.75	53.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 73.44	40.63, 75.00
Min, Max	31.3, 93.8	15.6, 96.9
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	6.06 (17.02)	5.81 (14.18)
Median	4.69	7.82
25th, 75th Percentile	-3.12, 12.51	-6.25, 15.63
Min, Max	-25.0, 50.0	-15.6, 28.1
Week 52		
n	18	14
Mean (SD)	63.12 (21.47)	57.82 (21.33)
Median	64.96	64.07
25th, 75th Percentile	50.00, 84.38	43.75, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.8, 90.6	3.1, 81.3
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	5.83 (16.82)	6.92 (23.93)
Median	6.25	6.25
25th, 75th Percentile	-6.25, 12.50	-6.25, 21.87
Min, Max	-25.0, 40.6	-25.0, 53.1
Difference in change from baseline (95%CI)		1.09 (-13.62, 15.80)
P-value ^b		0.8806
Hedges'g (95% CI) ^c		0.05 (-0.65, 0.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	24	27
Mean (SD)	56.42 (21.01)	58.34 (18.87)
Median	56.26	56.25
25th, 75th Percentile	37.51, 73.44	40.63, 75.00
Min, Max	21.9, 93.8	28.1, 93.8
Week 26		
n	24	26
Mean (SD)	57.29 (18.38)	62.54 (17.57)
Median	56.26	62.51

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.76, 68.75	50.00, 75.00
Min, Max	25.0, 87.5	18.8, 92.9
Change from baseline to Week 26 ^a		
n	24	26
Mean (SD)	0.87 (16.28)	4.00 (14.85)
Median	0.00	3.13
25th, 75th Percentile	-7.81, 10.94	0.00, 9.37
Min, Max	-31.3, 34.4	-28.1, 37.5
Week 52		
n	24	25
Mean (SD)	63.41 (17.00)	59.52 (17.71)
Median	64.07	62.50
25th, 75th Percentile	48.44, 76.57	53.13, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	37.5, 90.6	21.9, 81.3
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	6.99 (16.07)	0.02 (14.68)
Median	7.37	0.00
25th, 75th Percentile	-4.69, 18.75	-9.37, 6.25
Min, Max	-28.1, 43.8	-28.1, 31.3
Difference in change from baseline (95%CI)		-6.98 (-15.82, 1.86)
P-value ^b		0.1191
Hedges'g (95% CI) ^c		-0.45 (-1.01, 0.12)
P-value for interaction term, treatment * [Baseline AGV]		0.5766

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	41	45
Mean (SD)	55.97 (22.54)	57.48 (20.64)
Median	53.13	59.38
25th, 75th Percentile	34.38, 75.00	40.63, 71.88
Min, Max	15.6, 93.8	0.0, 100.0
Week 26		
n	40	42
Mean (SD)	58.52 (19.75)	60.91 (19.22)
Median	65.63	60.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 71.88	50.00, 71.88
Min, Max	18.8, 93.8	15.6, 96.9
Change from baseline to Week 26 ^a		
n	40	42
Mean (SD)	1.85 (17.87)	3.12 (16.77)
Median	3.12	4.02
25th, 75th Percentile	-7.81, 12.50	-3.12, 12.50
Min, Max	-34.4, 50.0	-53.1, 37.5
Week 52		
n	40	43
Mean (SD)	61.33 (24.15)	58.94 (14.08)
Median	64.07	59.38
25th, 75th Percentile	43.76, 82.82	50.00, 71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 93.8	25.0, 81.3
Change from baseline to Week 52 ^a		
n	40	43
Mean (SD)	5.06 (18.38)	0.97 (17.82)
Median	6.25	-3.12
25th, 75th Percentile	-10.94, 18.75	-9.37, 9.38
Min, Max	-28.1, 43.8	-37.5, 53.1
Difference in change from baseline (95%CI)		-4.09 (-12.00, 3.82)
P-value ^b		0.3064
Hedges'g (95% CI) ^c		-0.22 (-0.66, 0.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Social Score		
Baseline		
n	20	15
Mean (SD)	60.27 (20.84)	57.09 (20.75)
Median	62.50	53.13
25th, 75th Percentile	40.63, 75.00	37.50, 75.00
Min, Max	28.1, 93.8	25.0, 90.6
Week 26		
n	19	13
Mean (SD)	60.04 (18.56)	58.35 (26.43)
Median	59.38	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 75.00	40.63, 78.13
Min, Max	28.1, 87.5	0.0, 92.9
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	-0.12 (11.77)	3.54 (20.68)
Median	0.00	3.13
25th, 75th Percentile	-9.37, 6.25	0.00, 12.50
Min, Max	-12.5, 34.4	-53.1, 34.4
Week 52		
n	20	14
Mean (SD)	60.72 (15.54)	54.05 (30.29)
Median	61.84	65.63
25th, 75th Percentile	50.01, 75.00	25.00, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.3.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	31.3, 87.5	0.0, 87.5
Change from baseline to Week 52 ^a		
n	20	14
Mean (SD)	0.45 (12.04)	-3.32 (20.72)
Median	0.45	1.78
25th, 75th Percentile	-7.81, 7.81	-9.37, 9.37
Min, Max	-21.9, 21.9	-53.1, 31.3
Difference in change from baseline (95%CI)		-3.76 (-16.64, 9.11)
P-value ^b		0.5481
Hedges'g (95% CI) ^c		-0.23 (-0.91, 0.46)
P-value for interaction term, treatment * [Ethnicity]		0.9642

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.003.007_qs_sum_ovr_qol_care_soc_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.4.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	33	31
Mean (SD)	67.33 (20.85)	66.74 (19.43)
Median	71.88	68.75
25th, 75th Percentile	56.25, 81.25	53.13, 81.25
Min, Max	15.6, 100.0	12.5, 100.0
Week 26		
n	31	28
Mean (SD)	68.55 (16.53)	62.97 (19.68)
Median	71.88	64.96

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.4.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 81.25	54.69, 76.57
Min, Max	28.1, 100.0	18.8, 96.9
Change from baseline to Week 26 ^a		
n	31	28
Mean (SD)	0.30 (13.19)	-2.55 (15.19)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 9.37	-15.63, 9.37
Min, Max	-34.4, 25.0	-46.9, 23.7
Week 52		
n	32	30
Mean (SD)	65.53 (23.36)	64.44 (21.30)
Median	71.88	68.75
25th, 75th Percentile	48.44, 84.38	53.13, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	9.4, 96.9	3.6, 96.9
Change from baseline to Week 52 ^a		
n	32	30
Mean (SD)	-2.73 (17.42)	-2.02 (17.71)
Median	-1.56	-1.56
25th, 75th Percentile	-15.63, 7.81	-9.37, 9.38
Min, Max	-34.4, 46.9	-62.1, 28.1
Difference in change from baseline (95%CI)		0.71 (-8.22, 9.64)
P-value ^b		0.8741
Hedges'g (95% CI) ^c		0.04 (-0.46, 0.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	28	29
Mean (SD)	53.91 (27.00)	63.90 (17.00)
Median	56.25	65.63
25th, 75th Percentile	37.51, 70.32	53.13, 75.00
Min, Max	3.1, 100.0	28.1, 100.0
Week 26		
n	28	27
Mean (SD)	60.72 (23.13)	72.46 (17.87)
Median	62.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 76.57	59.38, 87.50
Min, Max	3.1, 100.0	34.4, 100.0
Change from baseline to Week 26 ^a		
n	28	27
Mean (SD)	6.81 (20.16)	6.71 (16.30)
Median	0.00	6.25
25th, 75th Percentile	-3.13, 14.07	-6.25, 15.62
Min, Max	-43.8, 71.9	-21.9, 50.0
Week 52		
n	28	27
Mean (SD)	60.27 (21.80)	66.32 (13.38)
Median	60.94	65.63
25th, 75th Percentile	50.00, 76.57	59.38, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.6, 100.0	34.4, 90.6
Change from baseline to Week 52 ^a		
n	28	27
Mean (SD)	6.36 (17.46)	1.04 (10.22)
Median	3.12	3.12
25th, 75th Percentile	-3.12, 12.50	-9.37, 6.25
Min, Max	-31.3, 65.6	-15.6, 28.1
Difference in change from baseline (95%CI)		-5.32 (-13.06, 2.42)
P-value ^b		0.1730
Hedges'g (95% CI) ^c		-0.36 (-0.90, 0.17)
P-value for interaction term, treatment * [Sex]		0.3156

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	24	31
Mean (SD)	64.98 (23.31)	65.22 (16.71)
Median	68.75	68.75
25th, 75th Percentile	48.44, 85.94	50.00, 78.13
Min, Max	12.5, 100.0	34.4, 100.0
Week 26		
n	24	28
Mean (SD)	64.72 (19.32)	69.44 (19.28)
Median	62.50	70.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	48.44, 81.25	56.25, 87.50
Min, Max	28.1, 100.0	18.8, 96.9
Change from baseline to Week 26 ^a		
n	24	28
Mean (SD)	-0.26 (17.04)	4.15 (16.88)
Median	0.00	4.69
25th, 75th Percentile	-6.25, 7.82	-3.12, 14.06
Min, Max	-43.8, 31.3	-46.9, 50.0
Week 52		
n	23	31
Mean (SD)	66.17 (24.36)	64.98 (19.04)
Median	71.88	68.75
25th, 75th Percentile	50.00, 84.38	56.25, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	9.4, 100.0	3.6, 96.9
Change from baseline to Week 52 ^a		
n	23	31
Mean (SD)	0.00 (14.66)	-0.24 (16.68)
Median	0.00	3.12
25th, 75th Percentile	-3.13, 9.37	-6.25, 9.38
Min, Max	-31.3, 31.3	-62.1, 28.1
Difference in change from baseline (95%CI)		-0.25 (-9.00, 8.51)
P-value ^b		0.9554
Hedges'g (95% CI) ^c		-0.02 (-0.55, 0.52)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	24	17
Mean (SD)	60.16 (24.58)	61.95 (20.80)
Median	62.50	62.50
25th, 75th Percentile	45.32, 76.57	53.13, 75.00
Min, Max	12.5, 100.0	12.5, 90.6
Week 26		
n	23	16
Mean (SD)	63.05 (21.91)	60.35 (19.46)
Median	68.75	60.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 81.25	48.44, 76.57
Min, Max	3.1, 93.8	28.1, 93.8
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	2.45 (13.49)	-2.73 (15.09)
Median	-3.13	-6.25
25th, 75th Percentile	-6.25, 12.50	-18.75, 10.94
Min, Max	-15.6, 40.6	-21.9, 25.0
Week 52		
n	24	16
Mean (SD)	57.69 (21.83)	63.09 (17.98)
Median	57.82	65.63
25th, 75th Percentile	43.76, 68.76	56.25, 73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	25.0, 96.9	9.4, 90.6
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	-2.47 (17.96)	0.00 (12.24)
Median	-3.13	0.00
25th, 75th Percentile	-10.94, 9.38	-9.37, 6.25
Min, Max	-34.4, 46.9	-18.8, 28.1
Difference in change from baseline (95%CI)		2.47 (-7.95, 12.89)
P-value ^b		0.6337
Hedges'g (95% CI) ^c		0.15 (-0.48, 0.78)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	13	12
Mean (SD)	56.01 (27.70)	70.58 (18.39)
Median	59.38	64.07
25th, 75th Percentile	46.88, 71.88	60.94, 85.94
Min, Max	3.1, 100.0	43.8, 100.0
Week 26		
n	12	11
Mean (SD)	68.49 (19.47)	73.58 (17.19)
Median	71.88	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	64.07, 76.57	59.38, 87.50
Min, Max	18.8, 100.0	43.8, 100.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	12.50 (20.90)	3.41 (16.44)
Median	6.25	0.00
25th, 75th Percentile	0.00, 17.19	-9.37, 12.50
Min, Max	-6.3, 71.9	-18.8, 40.6
Week 52		
n	13	10
Mean (SD)	67.55 (20.35)	70.00 (14.30)
Median	75.00	68.76
25th, 75th Percentile	59.38, 78.13	59.38, 84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.6, 96.9	50.0, 90.6
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	11.54 (20.39)	-2.50 (12.04)
Median	6.25	-6.25
25th, 75th Percentile	0.00, 12.50	-9.37, 3.12
Min, Max	-9.4, 65.6	-12.5, 28.1
Difference in change from baseline (95%CI)		-14.04 (-29.18, 1.10)
P-value ^b		0.0675
Hedges'g (95% CI) ^c		-0.78 (-1.63, 0.08)
P-value for interaction term, treatment * [Age at Baseline]		0.1388

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.4.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	48	48
Mean (SD)	62.89 (24.48)	63.67 (17.86)
Median	67.19	67.19
25th, 75th Percentile	46.88, 82.82	51.57, 75.00
Min, Max	3.1, 100.0	12.5, 100.0
Week 26		
n	46	44
Mean (SD)	66.03 (18.78)	66.13 (19.34)
Median	68.75	70.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.004.003_qs_sum_ovr_qol_care_emo_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.4.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 81.25	54.69, 79.69
Min, Max	18.8, 100.0	18.8, 96.9
Change from baseline to Week 26 ^a		
n	46	44
Mean (SD)	2.72 (18.37)	2.78 (16.27)
Median	0.00	4.69
25th, 75th Percentile	-6.25, 9.38	-6.25, 12.50
Min, Max	-43.8, 71.9	-46.9, 50.0
Week 52		
n	47	47
Mean (SD)	63.30 (23.25)	63.87 (18.44)
Median	65.63	65.63
25th, 75th Percentile	50.00, 84.38	56.25, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	9.4, 100.0	3.6, 96.9
Change from baseline to Week 52 ^a		
n	47	47
Mean (SD)	-0.13 (18.87)	0.44 (15.61)
Median	0.00	3.12
25th, 75th Percentile	-9.37, 9.37	-6.25, 9.38
Min, Max	-34.4, 65.6	-62.1, 28.1
Difference in change from baseline (95%CI)		0.57 (-6.52, 7.66)
P-value ^b		0.8736
Hedges'g (95% CI) ^c		0.03 (-0.37, 0.44)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	13	12
Mean (SD)	54.81 (24.99)	72.14 (18.72)
Median	56.25	68.75
25th, 75th Percentile	40.63, 65.63	60.94, 87.51
Min, Max	12.5, 100.0	43.8, 100.0
Week 26		
n	13	11
Mean (SD)	60.58 (24.79)	73.58 (18.51)
Median	71.88	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 75.00	59.38, 93.75
Min, Max	3.1, 100.0	43.8, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	5.77 (11.33)	-1.14 (16.73)
Median	6.25	-3.12
25th, 75th Percentile	-3.13, 12.50	-12.50, 3.12
Min, Max	-9.4, 25.0	-18.8, 40.6
Week 52		
n	13	10
Mean (SD)	62.26 (20.98)	72.19 (13.54)
Median	59.38	67.19
25th, 75th Percentile	53.13, 78.13	65.63, 87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	25.0, 96.9	50.0, 90.6
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	7.45 (12.60)	-5.31 (7.23)
Median	6.25	-6.25
25th, 75th Percentile	0.00, 12.50	-12.50, 3.12
Min, Max	-9.4, 37.5	-15.6, 3.1
Difference in change from baseline (95%CI)		-12.77 (-22.07, -3.46)
P-value ^b		0.0095
Hedges'g (95% CI) ^c		-1.16 (-2.04, -0.25)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.0832

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	10	15
Mean (SD)	64.69 (23.57)	60.21 (18.05)
Median	57.82	62.50
25th, 75th Percentile	46.88, 87.50	43.75, 75.00
Min, Max	34.4, 100.0	28.1, 90.6
Week 26		
n	10	12
Mean (SD)	58.13 (21.15)	70.57 (16.18)
Median	48.44	73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.004.005_qs_sum_ovr_qol_care_emo_bhgt_301_fas.pdf+rtf

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 71.88	56.25, 82.82
Min, Max	34.4, 100.0	43.8, 93.8
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-6.56 (16.50)	8.07 (18.63)
Median	0.00	9.38
25th, 75th Percentile	-15.62, 3.12	-3.13, 15.62
Min, Max	-43.8, 12.5	-18.8, 50.0
Week 52		
n	10	13
Mean (SD)	60.32 (23.76)	63.46 (12.33)
Median	53.13	65.63
25th, 75th Percentile	50.00, 84.38	56.25, 71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	28.1, 96.9	37.5, 84.4
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	-4.38 (10.64)	3.12 (11.55)
Median	-3.12	3.12
25th, 75th Percentile	-6.25, 0.00	0.00, 3.13
Min, Max	-31.3, 9.4	-18.8, 28.1
Difference in change from baseline (95%CI)		7.50 (-2.27, 17.27)
P-value ^b		0.1255
Hedges'g (95% CI) ^c		0.65 (-0.21, 1.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	24	18
Mean (SD)	63.54 (25.58)	63.02 (15.21)
Median	67.19	64.07
25th, 75th Percentile	48.44, 82.82	50.00, 75.00
Min, Max	3.1, 100.0	37.5, 84.4
Week 26		
n	22	18
Mean (SD)	71.31 (16.67)	57.64 (16.78)
Median	73.44	57.82

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 81.25	53.13, 71.88
Min, Max	37.5, 100.0	18.8, 81.3
Change from baseline to Week 26 ^a		
n	22	18
Mean (SD)	6.82 (17.88)	-5.38 (16.00)
Median	0.00	-6.25
25th, 75th Percentile	-3.13, 12.50	-15.62, 6.25
Min, Max	-9.4, 71.9	-46.9, 18.8
Week 52		
n	23	18
Mean (SD)	68.62 (22.76)	57.15 (18.36)
Median	75.00	60.94
25th, 75th Percentile	56.25, 84.38	50.00, 65.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	9.4, 100.0	3.6, 81.3
Change from baseline to Week 52 ^a		
n	23	18
Mean (SD)	3.94 (18.10)	-5.88 (19.13)
Median	3.12	-1.57
25th, 75th Percentile	-3.13, 9.37	-12.50, 3.13
Min, Max	-34.4, 65.6	-62.1, 28.1
Difference in change from baseline (95%CI)		-9.82 (-21.63, 1.99)
P-value ^b		0.1007
Hedges'g (95% CI) ^c		-0.52 (-1.14, 0.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	19	22
Mean (SD)	55.27 (25.94)	68.47 (20.34)
Median	59.38	70.32
25th, 75th Percentile	28.13, 75.00	56.25, 81.25
Min, Max	12.5, 93.8	12.5, 100.0
Week 26		
n	19	20
Mean (SD)	58.72 (23.58)	72.53 (20.17)
Median	62.50	76.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 81.25	64.29, 87.50
Min, Max	3.1, 87.5	28.1, 100.0
Change from baseline to Week 26 ^a		
n	19	20
Mean (SD)	3.45 (17.27)	5.18 (14.37)
Median	0.00	4.69
25th, 75th Percentile	-6.25, 18.75	-3.12, 14.07
Min, Max	-34.4, 40.6	-18.8, 40.6
Week 52		
n	19	21
Mean (SD)	58.39 (24.00)	71.64 (17.75)
Median	62.50	75.00
25th, 75th Percentile	34.38, 75.00	68.75, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.6, 93.8	9.4, 96.9
Change from baseline to Week 52 ^a		
n	19	21
Mean (SD)	3.13 (22.34)	2.15 (11.78)
Median	0.00	3.12
25th, 75th Percentile	-18.75, 21.88	-6.25, 12.50
Min, Max	-31.3, 46.9	-15.6, 28.1
Difference in change from baseline (95%CI)		-0.98 (-12.75, 10.79)
P-value ^b		0.8658
Hedges'g (95% CI) ^c		-0.05 (-0.67, 0.57)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	8	5
Mean (SD)	63.67 (21.32)	75.63 (16.74)
Median	68.75	65.63
25th, 75th Percentile	60.94, 73.44	62.50, 90.63
Min, Max	15.6, 87.5	62.5, 96.9
Week 26		
n	8	5
Mean (SD)	69.93 (14.07)	76.88 (21.15)
Median	71.88	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.004.005_qs_sum_ovr_qol_care_emo_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 79.69	75.00, 93.75
Min, Max	40.6, 84.4	43.8, 96.9
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	6.25 (11.81)	1.25 (12.22)
Median	7.82	3.12
25th, 75th Percentile	-4.69, 14.07	0.00, 9.37
Min, Max	-9.4, 25.0	-18.8, 12.5
Week 52		
n	8	5
Mean (SD)	61.72 (17.34)	73.13 (20.08)
Median	65.63	81.25
25th, 75th Percentile	53.13, 75.01	53.13, 90.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	28.1, 78.1	50.0, 90.6
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-1.95 (12.15)	-2.50 (11.13)
Median	0.01	-6.25
25th, 75th Percentile	-10.94, 7.82	-9.37, 0.00
Min, Max	-21.9, 12.5	-12.5, 15.6
Difference in change from baseline (95%CI)		-0.55 (-15.35, 14.25)
P-value ^b		0.9363
Hedges'g (95% CI) ^c		-0.04 (-1.16, 1.08)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.2433

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	19	19
Mean (SD)	64.31 (24.17)	68.09 (13.76)
Median	62.50	68.75
25th, 75th Percentile	46.88, 84.38	62.50, 78.13
Min, Max	12.5, 100.0	34.4, 84.4
Week 26		
n	19	15
Mean (SD)	67.93 (23.10)	68.04 (18.51)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 84.38	56.25, 81.25
Min, Max	3.1, 100.0	18.8, 90.6
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	3.62 (14.78)	-0.30 (17.53)
Median	6.25	6.25
25th, 75th Percentile	-6.25, 12.50	-6.25, 12.50
Min, Max	-21.9, 40.6	-46.9, 18.8
Week 52		
n	18	18
Mean (SD)	61.11 (26.06)	65.83 (20.69)
Median	59.38	67.19
25th, 75th Percentile	50.00, 78.13	59.38, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	9.4, 96.9	3.6, 96.9
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-4.69 (15.91)	-1.89 (18.95)
Median	-4.69	1.56
25th, 75th Percentile	-12.50, 3.13	-9.37, 12.50
Min, Max	-34.4, 21.9	-62.1, 28.1
Difference in change from baseline (95%CI)		2.80 (-9.05, 14.65)
P-value ^b		0.6340
Hedges'g (95% CI) ^c		0.16 (-0.50, 0.81)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	18	14
Mean (SD)	54.86 (25.75)	61.39 (25.64)
Median	56.25	65.63
25th, 75th Percentile	40.63, 71.88	43.75, 81.25
Min, Max	3.1, 100.0	12.5, 100.0
Week 26		
n	16	14
Mean (SD)	65.82 (18.31)	64.07 (23.12)
Median	71.88	68.76

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.69, 73.44	43.75, 78.13
Min, Max	28.1, 100.0	28.1, 100.0
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	10.74 (19.53)	2.68 (13.91)
Median	3.13	1.56
25th, 75th Percentile	-1.56, 17.19	-6.25, 15.62
Min, Max	-3.1, 71.9	-18.8, 25.0
Week 52		
n	18	14
Mean (SD)	64.93 (20.83)	62.28 (21.84)
Median	67.19	64.07
25th, 75th Percentile	46.88, 78.13	53.13, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	28.1, 96.9	9.4, 90.6
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	10.07 (21.15)	0.89 (15.47)
Median	4.69	1.57
25th, 75th Percentile	0.00, 12.50	-9.37, 9.38
Min, Max	-25.0, 65.6	-31.3, 28.1
Difference in change from baseline (95%CI)		-9.18 (-22.93, 4.58)
P-value ^b		0.1833
Hedges'g (95% CI) ^c		-0.47 (-1.18, 0.24)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	24	27
Mean (SD)	63.41 (24.23)	65.51 (16.66)
Median	68.75	65.63
25th, 75th Percentile	48.44, 82.82	50.00, 78.13
Min, Max	12.5, 93.8	40.6, 100.0
Week 26		
n	24	26
Mean (SD)	61.72 (19.20)	69.30 (17.89)
Median	62.50	70.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.32, 78.13	56.25, 81.25
Min, Max	18.8, 93.8	34.4, 96.9
Change from baseline to Week 26 ^a		
n	24	26
Mean (SD)	-1.69 (15.72)	2.95 (17.20)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 4.69	-6.25, 9.37
Min, Max	-43.8, 25.0	-18.8, 50.0
Week 52		
n	24	25
Mean (SD)	63.15 (21.98)	66.68 (13.23)
Median	65.63	68.75
25th, 75th Percentile	50.00, 81.26	56.25, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.6, 100.0	34.4, 90.6
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	-0.26 (14.57)	-0.45 (10.55)
Median	1.56	0.00
25th, 75th Percentile	-7.81, 7.81	-6.25, 3.13
Min, Max	-31.3, 31.3	-18.8, 28.1
Difference in change from baseline (95%CI)		-0.19 (-7.47, 7.10)
P-value ^b		0.9592
Hedges'g (95% CI) ^c		-0.01 (-0.57, 0.55)
P-value for interaction term, treatment * [Baseline AGV]		0.2879

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	41	45
Mean (SD)	61.89 (24.97)	66.95 (16.93)
Median	65.63	68.75
25th, 75th Percentile	46.88, 81.25	56.25, 78.13
Min, Max	3.1, 100.0	28.1, 100.0
Week 26		
n	40	42
Mean (SD)	66.02 (21.27)	69.06 (16.81)
Median	70.32	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	51.57, 81.25	59.38, 81.25
Min, Max	3.1, 100.0	28.1, 100.0
Change from baseline to Week 26 ^a		
n	40	42
Mean (SD)	3.98 (18.70)	1.72 (13.87)
Median	0.00	0.00
25th, 75th Percentile	-3.13, 12.50	-6.25, 12.50
Min, Max	-43.8, 71.9	-21.9, 40.6
Week 52		
n	40	43
Mean (SD)	64.14 (24.62)	67.69 (12.93)
Median	68.75	68.75
25th, 75th Percentile	48.44, 84.38	59.38, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	9.4, 100.0	37.5, 90.6
Change from baseline to Week 52 ^a		
n	40	43
Mean (SD)	1.64 (19.42)	0.47 (12.84)
Median	0.00	3.12
25th, 75th Percentile	-7.81, 9.37	-9.37, 6.25
Min, Max	-34.4, 65.6	-31.3, 28.1
Difference in change from baseline (95%CI)		-1.17 (-8.44, 6.09)
P-value ^b		0.7482
Hedges'g (95% CI) ^c		-0.07 (-0.50, 0.36)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Emotional Score		
Baseline		
n	20	15
Mean (SD)	59.69 (24.39)	60.63 (21.52)
Median	62.51	62.50
25th, 75th Percentile	43.76, 76.57	43.75, 81.25
Min, Max	12.5, 100.0	12.5, 90.6
Week 26		
n	19	13
Mean (SD)	62.34 (17.84)	62.98 (25.89)
Median	62.50	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.004.007_qs_sum_ovr_qol_care_emo_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.4.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 75.00	50.00, 87.50
Min, Max	28.1, 84.4	18.8, 93.8
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	2.14 (13.22)	2.88 (23.12)
Median	0.00	3.12
25th, 75th Percentile	-6.25, 12.50	-6.25, 15.63
Min, Max	-21.9, 31.3	-46.9, 50.0
Week 52		
n	20	14
Mean (SD)	60.94 (18.35)	58.07 (27.55)
Median	62.50	60.94
25th, 75th Percentile	50.00, 76.57	50.00, 78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.4.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	28.1, 93.8	3.6, 96.9
Change from baseline to Week 52 ^a		
n	20	14
Mean (SD)	1.25 (14.81)	-3.76 (19.28)
Median	1.56	0.00
25th, 75th Percentile	-6.25, 10.94	-6.25, 9.38
Min, Max	-31.3, 37.5	-62.1, 15.6
Difference in change from baseline (95%CI)		-5.01 (-16.92, 6.89)
P-value ^b		0.3975
Hedges'g (95% CI) ^c		-0.29 (-0.98, 0.40)
P-value for interaction term, treatment * [Ethnicity]		0.5725

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.5.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	32	30
Mean (SD)	43.22 (22.00)	48.06 (18.94)
Median	45.00	48.75
25th, 75th Percentile	27.50, 55.00	32.50, 62.50
Min, Max	0.0, 100.0	12.5, 82.5
Week 26		
n	31	26
Mean (SD)	41.29 (13.93)	50.38 (23.63)
Median	42.50	51.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.5.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 52.50	35.00, 65.00
Min, Max	5.0, 70.0	5.0, 97.5
Change from baseline to Week 26 ^a		
n	30	26
Mean (SD)	-1.50 (19.96)	1.86 (13.68)
Median	1.25	1.67
25th, 75th Percentile	-10.00, 7.50	-12.50, 10.00
Min, Max	-62.5, 25.0	-22.5, 32.5
Week 52		
n	32	30
Mean (SD)	40.01 (20.70)	46.82 (20.36)
Median	39.45	50.00
25th, 75th Percentile	27.50, 52.50	37.50, 57.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 75.0	0.0, 90.0
Change from baseline to Week 52 ^a		
n	31	29
Mean (SD)	-1.70 (18.97)	0.79 (11.39)
Median	0.00	-2.50
25th, 75th Percentile	-6.11, 7.50	-7.50, 7.50
Min, Max	-65.0, 52.5	-20.0, 27.5
Difference in change from baseline (95%CI)		2.50 (-5.56, 10.55)
P-value ^b		0.5359
Hedges'g (95% CI) ^c		0.16 (-0.35, 0.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	28	27
Mean (SD)	46.48 (20.68)	47.87 (16.56)
Median	43.75	47.50
25th, 75th Percentile	27.50, 62.50	35.00, 60.00
Min, Max	10.0, 92.5	20.0, 80.0
Week 26		
n	28	25
Mean (SD)	51.37 (15.73)	48.18 (21.31)
Median	53.75	46.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	41.25, 60.42	35.00, 60.00
Min, Max	22.5, 87.5	7.5, 90.0
Change from baseline to Week 26 ^a		
n	28	24
Mean (SD)	4.89 (21.08)	1.64 (21.99)
Median	4.31	1.25
25th, 75th Percentile	-5.00, 13.75	-11.25, 10.00
Min, Max	-47.5, 60.0	-37.5, 60.0
Week 52		
n	28	27
Mean (SD)	49.11 (18.85)	47.69 (18.62)
Median	48.75	45.00
25th, 75th Percentile	35.00, 57.50	35.00, 67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.0, 95.0	7.5, 77.5
Change from baseline to Week 52 ^a		
n	28	25
Mean (SD)	2.63 (19.37)	0.10 (20.82)
Median	0.00	-2.50
25th, 75th Percentile	-7.50, 7.50	-5.00, 7.50
Min, Max	-22.5, 65.0	-55.0, 47.5
Difference in change from baseline (95%CI)		-2.53 (-13.61, 8.56)
P-value ^b		0.6489
Hedges'g (95% CI) ^c		-0.12 (-0.66, 0.42)
P-value for interaction term, treatment * [Sex]		0.4585

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	23	28
Mean (SD)	38.70 (19.27)	47.23 (19.93)
Median	45.00	48.75
25th, 75th Percentile	20.00, 52.50	26.25, 62.50
Min, Max	0.0, 70.0	12.5, 80.0
Week 26		
n	24	25
Mean (SD)	44.58 (15.12)	47.08 (23.91)
Median	47.50	46.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 55.00	25.00, 65.00
Min, Max	17.5, 80.0	7.5, 90.0
Change from baseline to Week 26 ^a		
n	23	24
Mean (SD)	5.54 (14.52)	-0.13 (15.62)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 7.50	-11.25, 8.75
Min, Max	-15.0, 60.0	-37.5, 32.5
Week 52		
n	23	31
Mean (SD)	40.71 (22.41)	44.67 (20.55)
Median	45.00	45.00
25th, 75th Percentile	17.50, 52.50	30.00, 60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 85.0	0.0, 77.5
Change from baseline to Week 52 ^a		
n	22	28
Mean (SD)	4.38 (18.74)	-1.26 (13.30)
Median	-1.25	-2.50
25th, 75th Percentile	-5.00, 7.50	-7.50, 2.50
Min, Max	-22.5, 65.0	-32.5, 27.5
Difference in change from baseline (95%CI)		-5.64 (-14.75, 3.47)
P-value ^b		0.2192
Hedges'g (95% CI) ^c		-0.35 (-0.91, 0.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	24	17
Mean (SD)	48.78 (22.67)	47.16 (15.14)
Median	43.75	45.00
25th, 75th Percentile	35.00, 62.50	35.00, 60.00
Min, Max	12.5, 100.0	25.0, 75.0
Week 26		
n	23	15
Mean (SD)	48.04 (16.06)	45.67 (22.67)
Median	45.00	45.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 62.50	35.00, 65.00
Min, Max	25.0, 87.5	5.0, 82.5
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	-0.54 (22.85)	-1.44 (19.41)
Median	0.00	2.50
25th, 75th Percentile	-12.50, 12.50	-20.00, 12.50
Min, Max	-62.5, 50.0	-32.5, 40.0
Week 52		
n	24	16
Mean (SD)	45.58 (16.74)	49.38 (17.95)
Median	41.25	47.50
25th, 75th Percentile	35.00, 55.00	38.75, 62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	20.0, 95.0	20.0, 80.0
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	-3.20 (14.45)	2.08 (18.89)
Median	-2.50	5.00
25th, 75th Percentile	-14.62, 3.75	-3.75, 10.42
Min, Max	-30.0, 35.0	-55.0, 40.0
Difference in change from baseline (95%CI)		5.28 (-5.40, 15.96)
P-value ^b		0.3231
Hedges'g (95% CI) ^c		0.32 (-0.32, 0.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	13	12
Mean (SD)	47.99 (21.11)	50.83 (16.56)
Median	52.50	50.00
25th, 75th Percentile	38.89, 57.50	43.75, 61.25
Min, Max	7.5, 87.5	20.0, 82.5
Week 26		
n	12	11
Mean (SD)	45.28 (16.28)	59.32 (16.05)
Median	48.75	55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 56.25	50.00, 60.00
Min, Max	5.0, 65.0	42.5, 97.5
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	-1.92 (25.95)	10.23 (19.73)
Median	1.81	7.50
25th, 75th Percentile	-12.50, 20.00	-2.50, 15.00
Min, Max	-60.0, 30.0	-12.5, 60.0
Week 52		
n	13	10
Mean (SD)	48.08 (22.55)	51.75 (18.45)
Median	50.00	51.25
25th, 75th Percentile	35.00, 70.00	35.00, 60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.005.002_qs_sum_ovr_qol_care_cop_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.5.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 75.0	25.0, 90.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	0.09 (26.40)	2.75 (20.39)
Median	5.00	-3.75
25th, 75th Percentile	-3.89, 7.50	-10.00, 15.00
Min, Max	-65.0, 52.5	-20.0, 47.5
Difference in change from baseline (95%CI)		2.66 (-18.34, 23.67)
P-value ^b		0.7945
Hedges'g (95% CI) ^c		0.11 (-0.72, 0.93)
P-value for interaction term, treatment * [Age at Baseline]		0.3407

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.5.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	47	45
Mean (SD)	44.38 (21.83)	47.15 (17.60)
Median	45.00	47.50
25th, 75th Percentile	27.50, 57.50	35.00, 60.00
Min, Max	0.0, 100.0	12.5, 80.0
Week 26		
n	46	40
Mean (SD)	45.92 (13.99)	46.48 (22.57)
Median	45.00	45.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 55.00	26.25, 61.25
Min, Max	17.5, 80.0	5.0, 90.0
Change from baseline to Week 26 ^a		
n	45	39
Mean (SD)	1.89 (20.58)	0.01 (16.97)
Median	5.00	0.00
25th, 75th Percentile	-5.00, 10.00	-12.50, 10.00
Min, Max	-62.5, 60.0	-37.5, 40.0
Week 52		
n	47	47
Mean (SD)	45.11 (20.41)	44.57 (18.65)
Median	45.00	45.00
25th, 75th Percentile	35.00, 57.50	30.00, 57.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 95.0	0.0, 77.5
Change from baseline to Week 52 ^a		
n	46	44
Mean (SD)	1.84 (16.54)	-1.24 (15.60)
Median	0.00	-2.50
25th, 75th Percentile	-5.00, 7.50	-6.25, 5.00
Min, Max	-30.0, 65.0	-55.0, 40.0
Difference in change from baseline (95%CI)		-3.08 (-9.82, 3.66)
P-value ^b		0.3667
Hedges'g (95% CI) ^c		-0.19 (-0.60, 0.23)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	13	12
Mean (SD)	46.07 (19.92)	51.04 (18.51)
Median	52.50	50.00
25th, 75th Percentile	35.00, 55.00	37.50, 62.50
Min, Max	7.5, 75.0	20.0, 82.5
Week 26		
n	13	11
Mean (SD)	46.60 (20.79)	59.55 (18.90)
Median	45.00	55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 58.33	45.00, 77.50
Min, Max	5.0, 87.5	35.0, 97.5
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	0.53 (21.41)	7.95 (20.79)
Median	0.00	2.50
25th, 75th Percentile	-2.50, 15.00	-7.50, 15.00
Min, Max	-60.0, 25.0	-12.5, 60.0
Week 52		
n	13	10
Mean (SD)	41.15 (19.99)	59.75 (18.72)
Median	47.50	60.00
25th, 75th Percentile	27.50, 55.00	45.00, 72.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 75.0	35.0, 90.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	-4.91 (26.50)	8.00 (17.90)
Median	-2.50	6.25
25th, 75th Percentile	-20.00, 2.50	-7.50, 15.00
Min, Max	-65.0, 52.5	-15.0, 47.5
Difference in change from baseline (95%CI)		12.91 (-7.39, 33.22)
P-value ^b		0.2001
Hedges'g (95% CI) ^c		0.54 (-0.31, 1.37)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.0572

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	10	15
Mean (SD)	45.14 (25.75)	51.94 (13.84)
Median	45.00	45.00
25th, 75th Percentile	25.00, 50.00	42.50, 60.00
Min, Max	10.0, 100.0	35.0, 80.0
Week 26		
n	10	11
Mean (SD)	49.00 (15.73)	46.82 (23.29)
Median	46.25	47.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.00, 57.50	25.00, 55.00
Min, Max	22.5, 80.0	7.5, 90.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	3.86 (30.35)	-1.74 (21.28)
Median	8.06	0.83
25th, 75th Percentile	0.00, 12.50	-20.00, 12.50
Min, Max	-62.5, 60.0	-37.5, 40.0
Week 52		
n	10	13
Mean (SD)	46.89 (20.07)	51.73 (17.66)
Median	42.50	50.00
25th, 75th Percentile	35.00, 60.00	40.00, 72.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.0, 85.0	25.0, 77.5
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	1.75 (25.91)	0.64 (15.28)
Median	-4.45	-2.50
25th, 75th Percentile	-7.50, 7.50	-5.00, 7.50
Min, Max	-30.0, 65.0	-20.0, 40.0
Difference in change from baseline (95%CI)		-1.11 (-19.06, 16.84)
P-value ^b		0.8990
Hedges'g (95% CI) ^c		-0.05 (-0.88, 0.77)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	23	17
Mean (SD)	49.48 (19.93)	43.24 (17.45)
Median	53.13	37.50
25th, 75th Percentile	27.50, 62.50	30.00, 55.00
Min, Max	17.5, 87.5	22.5, 75.0
Week 26		
n	22	18
Mean (SD)	47.99 (15.77)	43.47 (19.54)
Median	52.50	43.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 57.50	35.00, 60.00
Min, Max	5.0, 75.0	5.0, 82.5
Change from baseline to Week 26 ^a		
n	21	17
Mean (SD)	-1.15 (21.80)	0.00 (17.94)
Median	-5.00	0.00
25th, 75th Percentile	-10.00, 7.50	-12.50, 10.00
Min, Max	-60.0, 50.0	-32.5, 32.5
Week 52		
n	23	18
Mean (SD)	47.23 (22.87)	41.10 (15.03)
Median	52.50	45.00
25th, 75th Percentile	35.00, 60.00	22.50, 50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 82.5	20.0, 72.5
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-0.08 (24.89)	-2.22 (16.94)
Median	-1.25	-2.50
25th, 75th Percentile	-15.00, 12.50	-5.00, 0.00
Min, Max	-65.0, 52.5	-55.0, 22.5
Difference in change from baseline (95%CI)		-2.14 (-16.41, 12.13)
P-value ^b		0.7626
Hedges'g (95% CI) ^c		-0.10 (-0.73, 0.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	19	20
Mean (SD)	43.29 (18.39)	46.00 (20.17)
Median	42.50	48.75
25th, 75th Percentile	35.00, 52.50	26.25, 63.75
Min, Max	12.5, 92.5	12.5, 75.0
Week 26		
n	19	17
Mean (SD)	44.61 (13.00)	53.68 (24.19)
Median	45.00	55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 55.00	35.00, 77.50
Min, Max	17.5, 65.0	15.0, 85.0
Change from baseline to Week 26 ^a		
n	19	17
Mean (SD)	1.32 (15.33)	6.03 (17.88)
Median	0.00	2.50
25th, 75th Percentile	-2.50, 7.50	-2.50, 10.00
Min, Max	-47.5, 25.0	-12.5, 60.0
Week 52		
n	19	21
Mean (SD)	43.68 (17.72)	45.95 (22.03)
Median	45.00	45.00
25th, 75th Percentile	32.50, 50.00	40.00, 60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	17.5, 95.0	0.0, 80.0
Change from baseline to Week 52 ^a		
n	19	19
Mean (SD)	0.39 (7.23)	2.11 (18.19)
Median	0.00	2.50
25th, 75th Percentile	-5.00, 2.50	-10.00, 15.00
Min, Max	-10.0, 22.5	-32.5, 47.5
Difference in change from baseline (95%CI)		1.71 (-7.57, 10.99)
P-value ^b		0.7066
Hedges'g (95% CI) ^c		0.12 (-0.52, 0.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	8	5
Mean (SD)	34.06 (25.18)	60.00 (14.47)
Median	40.00	60.00
25th, 75th Percentile	10.00, 50.00	50.00, 62.50
Min, Max	0.0, 72.5	45.0, 82.5
Week 26		
n	8	5
Mean (SD)	40.63 (20.91)	60.88 (22.15)
Median	35.00	52.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	28.75, 45.00	46.88, 65.00
Min, Max	20.0, 87.5	42.5, 97.5
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	6.56 (15.64)	0.88 (11.31)
Median	7.50	2.50
25th, 75th Percentile	-5.00, 20.00	-7.50, 7.50
Min, Max	-17.5, 25.0	-13.1, 15.0
Week 52		
n	8	5
Mean (SD)	33.75 (17.58)	63.00 (19.64)
Median	38.75	62.50
25th, 75th Percentile	18.75, 48.75	60.00, 67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	5.0, 52.5	35.0, 90.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	-0.31 (12.99)	3.00 (11.10)
Median	3.75	5.00
25th, 75th Percentile	-8.75, 7.50	2.50, 7.50
Min, Max	-22.5, 15.0	-15.0, 15.0
Difference in change from baseline (95%CI)		3.31 (-12.16, 18.79)
P-value ^b		0.6467
Hedges'g (95% CI) ^c		0.25 (-0.88, 1.37)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.9526

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	19	19
Mean (SD)	42.31 (22.42)	49.34 (18.20)
Median	45.00	50.00
25th, 75th Percentile	22.50, 52.50	35.00, 62.50
Min, Max	10.0, 100.0	17.5, 80.0
Week 26		
n	19	13
Mean (SD)	43.33 (15.28)	53.08 (23.46)
Median	45.00	52.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.50, 57.50	42.50, 65.00
Min, Max	20.0, 70.0	12.5, 90.0
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	1.02 (17.97)	4.81 (18.86)
Median	5.00	10.00
25th, 75th Percentile	-5.00, 12.50	-2.50, 12.50
Min, Max	-62.5, 20.0	-32.5, 32.5
Week 52		
n	18	18
Mean (SD)	40.22 (19.02)	44.15 (19.04)
Median	38.20	45.00
25th, 75th Percentile	22.50, 55.00	30.00, 57.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	12.5, 75.0	0.0, 77.5
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-1.67 (16.65)	-4.04 (17.24)
Median	-3.20	-5.00
25th, 75th Percentile	-7.50, 2.50	-10.00, 5.00
Min, Max	-30.0, 52.5	-55.0, 22.5
Difference in change from baseline (95%CI)		-2.38 (-13.86, 9.11)
P-value ^b		0.6767
Hedges'g (95% CI) ^c		-0.14 (-0.79, 0.52)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	18	14
Mean (SD)	48.09 (23.56)	42.14 (19.34)
Median	52.82	41.25
25th, 75th Percentile	27.50, 65.00	25.00, 55.00
Min, Max	7.5, 87.5	12.5, 75.0
Week 26		
n	16	14
Mean (SD)	50.16 (21.30)	48.21 (23.28)
Median	55.00	48.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	36.25, 61.25	27.50, 65.00
Min, Max	5.0, 87.5	5.0, 80.0
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	2.97 (27.42)	6.07 (22.23)
Median	0.00	6.25
25th, 75th Percentile	-7.50, 22.50	-10.00, 10.00
Min, Max	-60.0, 60.0	-25.0, 60.0
Week 52		
n	18	14
Mean (SD)	47.58 (21.50)	48.75 (19.18)
Median	48.75	47.50
25th, 75th Percentile	35.00, 57.50	37.50, 67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 85.0	20.0, 75.0
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	-0.51 (25.41)	6.61 (21.87)
Median	1.25	1.25
25th, 75th Percentile	-14.24, 7.50	-2.50, 22.50
Min, Max	-65.0, 65.0	-32.5, 47.5
Difference in change from baseline (95%CI)		7.12 (-10.30, 24.54)
P-value ^b		0.4105
Hedges'g (95% CI) ^c		0.29 (-0.41, 0.99)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	23	24
Mean (SD)	44.13 (18.94)	50.28 (16.26)
Median	42.50	48.75
25th, 75th Percentile	32.50, 55.00	42.09, 61.25
Min, Max	0.0, 92.5	22.5, 82.5
Week 26		
n	24	24
Mean (SD)	45.52 (10.58)	47.89 (21.89)
Median	45.00	45.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 52.50	35.00, 57.50
Min, Max	25.0, 75.0	7.5, 97.5
Change from baseline to Week 26 ^a		
n	23	23
Mean (SD)	1.09 (17.85)	-2.60 (13.98)
Median	5.00	0.00
25th, 75th Percentile	-10.00, 7.50	-12.50, 5.00
Min, Max	-47.5, 50.0	-37.5, 27.5
Week 52		
n	24	25
Mean (SD)	44.79 (20.43)	48.60 (20.26)
Median	45.00	50.00
25th, 75th Percentile	37.50, 51.25	42.50, 60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.5.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 95.0	7.5, 90.0
Change from baseline to Week 52 ^a		
n	23	22
Mean (SD)	2.61 (15.53)	0.27 (9.45)
Median	0.00	1.25
25th, 75th Percentile	-5.00, 7.50	-7.50, 7.50
Min, Max	-22.5, 45.0	-17.5, 20.0
Difference in change from baseline (95%CI)		-2.34 (-10.07, 5.39)
P-value ^b		0.5426
Hedges'g (95% CI) ^c		-0.18 (-0.76, 0.41)
P-value for interaction term, treatment * [Baseline AGV]		0.4528

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	40	43
Mean (SD)	43.91 (18.34)	49.71 (18.27)
Median	42.50	50.00
25th, 75th Percentile	27.50, 56.25	37.50, 65.00
Min, Max	12.5, 87.5	12.5, 82.5
Week 26		
n	40	39
Mean (SD)	47.21 (14.95)	50.75 (23.08)
Median	47.50	47.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	38.75, 56.25	35.00, 67.50
Min, Max	5.0, 80.0	5.0, 97.5
Change from baseline to Week 26 ^a		
n	39	38
Mean (SD)	3.51 (19.82)	2.29 (17.08)
Median	5.00	3.75
25th, 75th Percentile	-5.00, 10.00	-10.00, 10.00
Min, Max	-60.0, 60.0	-32.5, 60.0
Week 52		
n	40	43
Mean (SD)	44.78 (20.33)	48.66 (18.22)
Median	45.00	47.50
25th, 75th Percentile	35.00, 57.50	40.00, 60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 85.0	10.0, 90.0
Change from baseline to Week 52 ^a		
n	39	41
Mean (SD)	2.18 (21.24)	0.55 (16.78)
Median	0.00	-2.50
25th, 75th Percentile	-5.00, 7.50	-5.00, 7.50
Min, Max	-65.0, 65.0	-55.0, 47.5
Difference in change from baseline (95%CI)		-1.63 (-10.13, 6.87)
P-value ^b		0.7034
Hedges'g (95% CI) ^c		-0.08 (-0.52, 0.35)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.005.007_qs_sum_ovr_qol_care_cop_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.5.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Coping Score		
Baseline		
n	20	14
Mean (SD)	46.41 (26.66)	42.62 (15.18)
Median	45.00	43.34
25th, 75th Percentile	30.00, 58.75	32.50, 57.50
Min, Max	0.0, 100.0	17.5, 62.5
Week 26		
n	19	12
Mean (SD)	43.68 (16.86)	44.58 (19.82)
Median	42.50	46.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 52.50	31.25, 60.00
Min, Max	17.5, 87.5	7.5, 75.0
Change from baseline to Week 26 ^a		
n	19	12
Mean (SD)	-2.37 (22.09)	0.07 (21.24)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 12.50	-12.50, 7.50
Min, Max	-62.5, 25.0	-37.5, 40.0
Week 52		
n	20	14
Mean (SD)	43.19 (20.47)	42.84 (22.78)
Median	45.00	48.75
25th, 75th Percentile	30.00, 53.75	22.50, 52.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.5.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	5.0, 95.0	0.0, 75.0
Change from baseline to Week 52 ^a		
n	20	13
Mean (SD)	-3.21 (13.90)	0.23 (15.24)
Median	0.00	-2.50
25th, 75th Percentile	-15.87, 6.25	-10.00, 5.00
Min, Max	-30.0, 15.0	-17.5, 40.0
Difference in change from baseline (95%CI)		3.45 (-7.04, 13.93)
P-value ^b		0.5077
Hedges'g (95% CI) ^c		0.23 (-0.47, 0.93)
P-value for interaction term, treatment * [Ethnicity]		0.5011

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	32	31
Mean (SD)	66.02 (25.89)	62.50 (30.36)
Median	71.88	62.50
25th, 75th Percentile	50.00, 87.50	50.00, 87.50
Min, Max	6.3, 100.0	0.0, 100.0
Week 26		
n	31	27
Mean (SD)	68.35 (26.95)	61.34 (30.82)
Median	75.00	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 93.75	43.75, 93.75
Min, Max	18.8, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	2.08 (23.06)	1.39 (22.42)
Median	0.00	6.25
25th, 75th Percentile	-12.50, 12.50	-6.25, 12.50
Min, Max	-50.0, 68.8	-75.0, 50.0
Week 52		
n	32	30
Mean (SD)	65.43 (28.57)	57.71 (29.67)
Median	71.88	56.25
25th, 75th Percentile	50.00, 90.63	37.50, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	31	30
Mean (SD)	-2.02 (21.00)	-4.79 (24.87)
Median	0.00	-6.25
25th, 75th Percentile	-12.50, 6.25	-18.75, 6.25
Min, Max	-43.8, 62.5	-75.0, 37.5
Difference in change from baseline (95%CI)		-2.78 (-14.56, 9.00)
P-value ^b		0.6391
Hedges'g (95% CI) ^c		-0.12 (-0.62, 0.38)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	28	29
Mean (SD)	50.89 (33.28)	67.46 (21.87)
Median	53.13	68.75
25th, 75th Percentile	21.88, 78.13	50.00, 81.25
Min, Max	0.0, 100.0	25.0, 100.0
Week 26		
n	28	26
Mean (SD)	54.91 (30.21)	62.74 (24.20)
Median	59.38	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 75.00	43.75, 87.50
Min, Max	0.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	28	26
Mean (SD)	4.02 (26.14)	-6.49 (13.52)
Median	6.25	-6.25
25th, 75th Percentile	-6.25, 15.63	-18.75, 0.00
Min, Max	-81.3, 50.0	-25.0, 25.0
Week 52		
n	28	26
Mean (SD)	53.57 (28.99)	69.23 (22.63)
Median	56.25	71.88
25th, 75th Percentile	37.50, 71.88	56.25, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	12.5, 100.0
Change from baseline to Week 52 ^a		
n	28	26
Mean (SD)	2.68 (24.97)	2.16 (13.10)
Median	0.00	3.13
25th, 75th Percentile	-9.38, 18.75	-6.25, 12.50
Min, Max	-62.5, 50.0	-25.0, 25.0
Difference in change from baseline (95%CI)		-0.52 (-11.36, 10.33)
P-value ^b		0.9241
Hedges'g (95% CI) ^c		-0.03 (-0.56, 0.51)
P-value for interaction term, treatment * [Sex]		0.7811

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	23	31
Mean (SD)	68.48 (27.40)	65.93 (27.80)
Median	75.00	75.00
25th, 75th Percentile	50.00, 93.75	50.00, 87.50
Min, Max	0.0, 100.0	0.0, 100.0
Week 26		
n	24	26
Mean (SD)	63.02 (24.99)	63.70 (29.10)
Median	68.75	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 84.38	43.75, 93.75
Min, Max	18.8, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	23	26
Mean (SD)	-4.89 (23.68)	-0.96 (21.92)
Median	0.00	0.00
25th, 75th Percentile	-18.75, 6.25	-6.25, 12.50
Min, Max	-81.3, 37.5	-75.0, 50.0
Week 52		
n	23	30
Mean (SD)	66.30 (27.42)	63.54 (27.62)
Median	68.75	68.75
25th, 75th Percentile	50.00, 87.50	43.75, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	22	30
Mean (SD)	-4.26 (25.54)	-1.46 (23.65)
Median	0.00	0.00
25th, 75th Percentile	-18.75, 6.25	-12.50, 12.50
Min, Max	-62.5, 50.0	-75.0, 37.5
Difference in change from baseline (95%CI)		2.80 (-10.99, 16.60)
P-value ^b		0.6849
Hedges'g (95% CI) ^c		0.11 (-0.44, 0.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	24	17
Mean (SD)	52.86 (30.51)	61.76 (25.38)
Median	56.25	62.50
25th, 75th Percentile	28.13, 75.00	50.00, 81.25
Min, Max	0.0, 100.0	6.3, 100.0
Week 26		
n	23	16
Mean (SD)	59.51 (35.10)	59.77 (27.29)
Median	75.00	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 87.50	37.50, 75.00
Min, Max	0.0, 100.0	12.5, 100.0
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	6.79 (25.21)	-2.73 (13.50)
Median	6.25	-3.13
25th, 75th Percentile	0.00, 12.50	-15.63, 6.25
Min, Max	-50.0, 68.8	-18.8, 25.0
Week 52		
n	24	16
Mean (SD)	53.91 (32.18)	64.06 (25.67)
Median	56.25	71.88
25th, 75th Percentile	34.38, 75.00	50.00, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 93.8
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	1.04 (21.70)	1.56 (14.34)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 9.38	-6.25, 15.63
Min, Max	-37.5, 62.5	-18.8, 25.0
Difference in change from baseline (95%CI)		0.52 (-11.98, 13.03)
P-value ^b		0.9332
Hedges'g (95% CI) ^c		0.03 (-0.61, 0.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.006.002_qs_sum_ovr_qol_care_bel_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	13	12
Mean (SD)	53.37 (32.64)	66.67 (26.43)
Median	50.00	62.50
25th, 75th Percentile	43.75, 87.50	62.50, 87.50
Min, Max	0.0, 100.0	12.5, 100.0
Week 26		
n	12	11
Mean (SD)	64.58 (25.88)	61.36 (26.19)
Median	68.75	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 81.25	43.75, 100.00
Min, Max	12.5, 100.0	37.5, 100.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	10.94 (21.51)	-5.68 (18.84)
Median	12.50	0.00
25th, 75th Percentile	3.13, 21.88	-25.00, 6.25
Min, Max	-37.5, 50.0	-31.3, 31.3
Week 52		
n	13	10
Mean (SD)	59.62 (25.72)	60.00 (29.93)
Median	50.00	59.38
25th, 75th Percentile	50.00, 81.25	37.50, 93.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 93.8	12.5, 100.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	6.25 (20.25)	-6.88 (18.74)
Median	6.25	-3.13
25th, 75th Percentile	0.00, 18.75	-6.25, 0.00
Min, Max	-37.5, 50.0	-50.0, 18.8
Difference in change from baseline (95%CI)		-13.13 (-30.29, 4.04)
P-value ^b		0.1266
Hedges'g (95% CI) ^c		-0.64 (-1.48, 0.21)
P-value for interaction term, treatment * [Age at Baseline]		0.3409

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	47	48
Mean (SD)	59.97 (30.41)	64.19 (27.67)
Median	62.50	71.88
25th, 75th Percentile	43.75, 87.50	46.88, 84.38
Min, Max	0.0, 100.0	0.0, 100.0
Week 26		
n	46	42
Mean (SD)	63.32 (27.75)	62.50 (28.52)
Median	71.88	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 87.50	43.75, 93.75
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	45	42
Mean (SD)	3.33 (24.52)	-0.74 (19.24)
Median	6.25	0.00
25th, 75th Percentile	-6.25, 12.50	-6.25, 6.25
Min, Max	-81.3, 68.8	-75.0, 50.0
Week 52		
n	47	46
Mean (SD)	61.44 (28.56)	61.28 (28.19)
Median	68.75	68.75
25th, 75th Percentile	50.00, 81.25	43.75, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	46	46
Mean (SD)	0.54 (23.53)	-2.31 (21.62)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 6.25	-12.50, 12.50
Min, Max	-62.5, 62.5	-75.0, 37.5
Difference in change from baseline (95%CI)		-2.85 (-12.21, 6.51)
P-value ^b		0.5463
Hedges'g (95% CI) ^c		-0.13 (-0.53, 0.28)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	13	12
Mean (SD)	55.29 (30.71)	67.71 (21.95)
Median	56.25	62.50
25th, 75th Percentile	25.00, 87.50	50.00, 87.50
Min, Max	12.5, 100.0	31.3, 100.0
Week 26		
n	13	11
Mean (SD)	57.21 (34.22)	60.23 (24.41)
Median	68.75	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 81.25	37.50, 75.00
Min, Max	0.0, 100.0	31.3, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	1.92 (24.92)	-9.09 (16.38)
Median	0.00	-18.75
25th, 75th Percentile	-12.50, 18.75	-18.75, 0.00
Min, Max	-37.5, 43.8	-25.0, 25.0
Week 52		
n	13	10
Mean (SD)	54.33 (31.70)	71.25 (20.03)
Median	56.25	71.88
25th, 75th Percentile	37.50, 81.25	56.25, 87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 93.8	37.5, 100.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	-0.96 (21.32)	1.88 (13.83)
Median	6.25	0.00
25th, 75th Percentile	-12.50, 18.75	-6.25, 6.25
Min, Max	-37.5, 25.0	-25.0, 25.0
Difference in change from baseline (95%CI)		2.84 (-13.34, 19.01)
P-value ^b		0.7190
Hedges'g (95% CI) ^c		0.15 (-0.68, 0.97)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.5807

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	10	15
Mean (SD)	70.63 (30.77)	61.67 (23.25)
Median	75.00	62.50
25th, 75th Percentile	56.25, 93.75	43.75, 75.00
Min, Max	0.0, 100.0	25.0, 100.0
Week 26		
n	10	12
Mean (SD)	61.25 (33.82)	64.58 (28.25)
Median	75.00	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 87.50	43.75, 96.88
Min, Max	0.0, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-9.38 (29.50)	1.04 (14.31)
Median	0.00	0.00
25th, 75th Percentile	-18.75, 0.00	-9.38, 12.50
Min, Max	-81.3, 25.0	-18.8, 25.0
Week 52		
n	10	13
Mean (SD)	58.13 (30.05)	67.79 (20.55)
Median	68.75	68.75
25th, 75th Percentile	31.25, 75.00	50.00, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	37.5, 100.0
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	-12.50 (24.65)	5.29 (14.40)
Median	-3.13	6.25
25th, 75th Percentile	-31.25, 0.00	-6.25, 18.75
Min, Max	-62.5, 18.8	-18.8, 25.0
Difference in change from baseline (95%CI)		17.79 (0.76, 34.82)
P-value ^b		0.0414
Hedges'g (95% CI) ^c		0.88 (0.01, 1.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	23	18
Mean (SD)	64.40 (30.59)	63.19 (27.78)
Median	68.75	68.75
25th, 75th Percentile	43.75, 87.50	43.75, 87.50
Min, Max	0.0, 100.0	12.5, 100.0
Week 26		
n	22	18
Mean (SD)	67.33 (24.92)	59.38 (25.30)
Median	75.00	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 87.50	43.75, 75.00
Min, Max	18.8, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	21	18
Mean (SD)	2.68 (20.21)	-3.82 (25.38)
Median	0.00	-3.13
25th, 75th Percentile	-12.50, 12.50	-12.50, 6.25
Min, Max	-37.5, 50.0	-75.0, 50.0
Week 52		
n	23	18
Mean (SD)	66.30 (25.68)	53.47 (30.26)
Median	68.75	56.25
25th, 75th Percentile	50.00, 93.75	25.00, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	0.00 (20.41)	-9.72 (26.19)
Median	0.00	-6.25
25th, 75th Percentile	-6.25, 6.25	-25.00, 6.25
Min, Max	-43.8, 50.0	-75.0, 37.5
Difference in change from baseline (95%CI)		-9.72 (-24.63, 5.19)
P-value ^b		0.1947
Hedges'g (95% CI) ^c		-0.41 (-1.04, 0.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.006.005_qs_sum_ovr_qol_care_bel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	19	22
Mean (SD)	46.05 (27.89)	66.19 (29.92)
Median	50.00	75.00
25th, 75th Percentile	25.00, 75.00	50.00, 87.50
Min, Max	0.0, 93.8	0.0, 100.0
Week 26		
n	19	19
Mean (SD)	52.96 (33.10)	63.16 (30.82)
Median	56.25	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.006.005_qs_sum_ovr_qol_care_bel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 87.50	37.50, 93.75
Min, Max	0.0, 100.0	12.5, 100.0
Change from baseline to Week 26 ^a		
n	19	19
Mean (SD)	6.91 (27.63)	0.00 (13.66)
Median	6.25	0.00
25th, 75th Percentile	-12.50, 18.75	-12.50, 6.25
Min, Max	-50.0, 68.8	-25.0, 25.0
Week 52		
n	19	20
Mean (SD)	51.97 (34.55)	67.50 (27.48)
Median	56.25	75.00
25th, 75th Percentile	25.00, 81.25	46.88, 87.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	19	20
Mean (SD)	5.92 (26.56)	2.81 (14.12)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 18.75	-6.25, 9.38
Min, Max	-37.5, 62.5	-25.0, 37.5
Difference in change from baseline (95%CI)		-3.11 (-17.18, 10.97)
P-value ^b		0.6541
Hedges'g (95% CI) ^c		-0.14 (-0.77, 0.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	8	5
Mean (SD)	59.38 (28.74)	75.00 (17.12)
Median	68.75	75.00
25th, 75th Percentile	43.75, 78.13	62.50, 81.25
Min, Max	6.3, 87.5	56.3, 100.0
Week 26		
n	8	4
Mean (SD)	69.53 (22.02)	60.94 (28.13)
Median	75.00	53.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 78.13	40.63, 81.25
Min, Max	18.8, 93.8	37.5, 100.0
Change from baseline to Week 26 ^a		
n	8	4
Mean (SD)	10.16 (16.68)	-18.75 (13.50)
Median	12.50	-21.88
25th, 75th Percentile	0.00, 12.50	-28.13, -9.38
Min, Max	-12.5, 43.8	-31.3, 0.0
Week 52		
n	8	5
Mean (SD)	62.50 (23.15)	67.50 (26.29)
Median	71.88	75.00
25th, 75th Percentile	46.88, 78.13	62.50, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.8, 87.5	25.0, 93.8
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	3.13 (12.94)	-7.50 (25.54)
Median	3.13	0.00
25th, 75th Percentile	-3.13, 9.38	-6.25, 0.00
Min, Max	-18.8, 25.0	-50.0, 18.8
Difference in change from baseline (95%CI)		-10.63 (-33.89, 12.64)
P-value ^b		0.3364
Hedges'g (95% CI) ^c		-0.53 (-1.66, 0.62)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.0886

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	19	19
Mean (SD)	67.43 (28.84)	68.09 (22.14)
Median	75.00	75.00
25th, 75th Percentile	50.00, 93.75	62.50, 81.25
Min, Max	6.3, 100.0	12.5, 100.0
Week 26		
n	19	15
Mean (SD)	65.79 (29.42)	62.08 (26.14)
Median	75.00	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 87.50	43.75, 87.50
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	-1.64 (19.86)	-6.25 (24.78)
Median	0.00	-6.25
25th, 75th Percentile	-18.75, 12.50	-18.75, 6.25
Min, Max	-37.5, 37.5	-75.0, 31.3
Week 52		
n	18	18
Mean (SD)	60.76 (35.73)	57.99 (29.61)
Median	71.88	71.88
25th, 75th Percentile	31.25, 93.75	25.00, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-8.33 (25.27)	-10.42 (25.46)
Median	-6.25	-6.25
25th, 75th Percentile	-18.75, 0.00	-18.75, 6.25
Min, Max	-62.5, 43.8	-75.0, 25.0
Difference in change from baseline (95%CI)		-2.08 (-19.27, 15.10)
P-value ^b		0.8069
Hedges'g (95% CI) ^c		-0.08 (-0.73, 0.57)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	18	14
Mean (SD)	49.65 (31.39)	62.50 (33.25)
Median	50.00	68.75
25th, 75th Percentile	25.00, 75.00	43.75, 93.75
Min, Max	0.0, 100.0	0.0, 100.0
Week 26		
n	16	14
Mean (SD)	62.89 (28.73)	61.61 (31.28)
Median	65.63	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 87.50	43.75, 93.75
Min, Max	18.8, 100.0	12.5, 100.0
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	13.67 (27.50)	-0.89 (10.92)
Median	9.38	0.00
25th, 75th Percentile	0.00, 28.13	-12.50, 6.25
Min, Max	-50.0, 68.8	-18.8, 12.5
Week 52		
n	18	14
Mean (SD)	59.38 (23.41)	66.52 (29.48)
Median	50.00	68.75
25th, 75th Percentile	43.75, 81.25	56.25, 93.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	25.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	18	14
Mean (SD)	9.72 (22.09)	4.02 (18.28)
Median	3.13	0.00
25th, 75th Percentile	0.00, 18.75	-6.25, 12.50
Min, Max	-31.3, 62.5	-31.3, 37.5
Difference in change from baseline (95%CI)		-5.70 (-20.64, 9.24)
P-value ^b		0.4416
Hedges'g (95% CI) ^c		-0.27 (-0.97, 0.43)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	23	27
Mean (SD)	59.24 (29.73)	63.89 (26.25)
Median	68.75	62.50
25th, 75th Percentile	37.50, 81.25	50.00, 93.75
Min, Max	0.0, 100.0	12.5, 100.0
Week 26		
n	24	24
Mean (SD)	58.33 (29.87)	62.24 (27.24)
Median	65.63	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 81.25	40.63, 84.38
Min, Max	0.0, 100.0	18.8, 100.0
Change from baseline to Week 26 ^a		
n	23	24
Mean (SD)	-0.54 (24.27)	-1.04 (18.67)
Median	6.25	0.00
25th, 75th Percentile	-12.50, 12.50	-18.75, 9.38
Min, Max	-81.3, 37.5	-25.0, 50.0
Week 52		
n	24	24
Mean (SD)	59.64 (28.79)	64.84 (24.02)
Median	68.75	65.63
25th, 75th Percentile	53.13, 75.00	46.88, 84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	12.5, 100.0
Change from baseline to Week 52 ^a		
n	23	24
Mean (SD)	-0.54 (19.39)	1.82 (15.36)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 12.50	-6.25, 9.38
Min, Max	-37.5, 50.0	-25.0, 37.5
Difference in change from baseline (95%CI)		2.37 (-7.89, 12.62)
P-value ^b		0.6444
Hedges'g (95% CI) ^c		0.13 (-0.44, 0.71)
P-value for interaction term, treatment * [Baseline AGV]		0.7004

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	40	45
Mean (SD)	59.22 (31.23)	66.81 (26.38)
Median	59.38	68.75
25th, 75th Percentile	43.75, 87.50	50.00, 87.50
Min, Max	0.0, 100.0	0.0, 100.0
Week 26		
n	40	40
Mean (SD)	63.44 (30.51)	65.31 (26.10)
Median	75.00	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.006.007_qs_sum_ovr_qol_care_bel_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.38, 87.50	43.75, 93.75
Min, Max	0.0, 100.0	12.5, 100.0
Change from baseline to Week 26 ^a		
n	39	40
Mean (SD)	4.33 (25.97)	-1.25 (14.24)
Median	6.25	0.00
25th, 75th Percentile	-6.25, 12.50	-12.50, 6.25
Min, Max	-81.3, 68.8	-31.3, 31.3
Week 52		
n	40	43
Mean (SD)	63.91 (31.63)	66.42 (23.98)
Median	68.75	68.75
25th, 75th Percentile	50.00, 93.75	50.00, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	12.5, 100.0
Change from baseline to Week 52 ^a		
n	39	43
Mean (SD)	3.69 (22.15)	-0.44 (18.27)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 12.50	-6.25, 12.50
Min, Max	-43.8, 62.5	-50.0, 37.5
Difference in change from baseline (95%CI)		-4.12 (-13.01, 4.77)
P-value ^b		0.3590
Hedges'g (95% CI) ^c		-0.20 (-0.64, 0.23)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.6.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Beliefs Score		
Baseline		
n	20	15
Mean (SD)	58.44 (29.05)	59.17 (26.92)
Median	68.75	62.50
25th, 75th Percentile	31.25, 81.25	43.75, 81.25
Min, Max	0.0, 93.8	6.3, 93.8
Week 26		
n	19	13
Mean (SD)	58.88 (26.38)	51.92 (30.34)
Median	68.75	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 75.00	31.25, 75.00
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	0.33 (21.19)	-6.25 (29.32)
Median	0.00	-6.25
25th, 75th Percentile	-12.50, 12.50	-18.75, 6.25
Min, Max	-37.5, 43.8	-75.0, 50.0
Week 52		
n	20	13
Mean (SD)	51.88 (21.94)	51.92 (34.08)
Median	53.13	50.00
25th, 75th Percentile	37.50, 71.88	37.50, 81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.6.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 87.5	0.0, 100.0
Change from baseline to Week 52 ^a		
n	20	13
Mean (SD)	-6.56 (23.34)	-5.29 (26.86)
Median	-3.13	0.00
25th, 75th Percentile	-18.75, 3.13	-18.75, 6.25
Min, Max	-62.5, 37.5	-75.0, 37.5
Difference in change from baseline (95%CI)		1.27 (-16.72, 19.27)
P-value ^b		0.8861
Hedges'g (95% CI) ^c		0.05 (-0.65, 0.75)
P-value for interaction term, treatment * [Ethnicity]		0.5521

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	32	30
Mean (SD)	76.72 (27.61)	68.08 (26.16)
Median	85.00	72.50
25th, 75th Percentile	72.50, 95.00	55.00, 90.00
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	31	27
Mean (SD)	76.45 (25.70)	70.37 (21.88)
Median	90.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 95.00	55.00, 85.00
Min, Max	0.0, 100.0	15.0, 100.0
Change from baseline to Week 26 ^a		
n	30	27
Mean (SD)	0.83 (14.74)	4.72 (20.99)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 20.00
Min, Max	-50.0, 25.0	-42.5, 50.0
Week 52		
n	31	30
Mean (SD)	74.52 (29.14)	70.50 (23.13)
Median	85.00	75.00
25th, 75th Percentile	60.00, 95.00	60.00, 85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	30	29
Mean (SD)	-4.17 (16.72)	1.47 (23.20)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 5.00	-5.00, 5.00
Min, Max	-70.0, 25.0	-62.5, 50.0
Difference in change from baseline (95%CI)		5.63 (-4.88, 16.15)
P-value ^b		0.2880
Hedges'g (95% CI) ^c		0.28 (-0.24, 0.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	28	28
Mean (SD)	58.39 (31.57)	70.71 (22.35)
Median	60.00	75.00
25th, 75th Percentile	32.50, 85.00	60.00, 87.50
Min, Max	0.0, 100.0	15.0, 100.0
Week 26		
n	28	27
Mean (SD)	60.80 (31.50)	77.59 (19.08)
Median	67.50	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 82.50	60.00, 95.00
Min, Max	0.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	28	26
Mean (SD)	2.41 (24.08)	5.38 (22.22)
Median	0.00	2.50
25th, 75th Percentile	-5.00, 15.00	-5.00, 15.00
Min, Max	-95.0, 40.0	-30.0, 60.0
Week 52		
n	28	27
Mean (SD)	61.61 (27.92)	77.96 (16.77)
Median	62.50	80.00
25th, 75th Percentile	50.00, 82.50	70.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	28	26
Mean (SD)	3.21 (21.70)	5.38 (16.49)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 10.00	-5.00, 10.00
Min, Max	-30.0, 75.0	-20.0, 45.0
Difference in change from baseline (95%CI)		2.17 (-8.41, 12.76)
P-value ^b		0.6824
Hedges'g (95% CI) ^c		0.11 (-0.42, 0.64)
P-value for interaction term, treatment * [Sex]		0.6434

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	23	29
Mean (SD)	75.00 (32.51)	70.78 (25.59)
Median	90.00	75.00
25th, 75th Percentile	70.00, 95.00	60.00, 90.00
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	24	27
Mean (SD)	72.08 (31.79)	74.81 (24.20)
Median	85.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.007.002_qs_sum_ovr_qol_care_fut_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	57.50, 95.00	55.00, 95.00
Min, Max	0.0, 100.0	15.0, 100.0
Change from baseline to Week 26 ^a		
n	23	26
Mean (SD)	-1.74 (22.84)	5.10 (19.45)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 5.00	-5.00, 15.00
Min, Max	-95.0, 40.0	-42.5, 45.0
Week 52		
n	23	31
Mean (SD)	76.09 (29.58)	75.00 (23.17)
Median	85.00	80.00
25th, 75th Percentile	60.00, 100.00	65.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	22	29
Mean (SD)	0.68 (19.17)	2.67 (20.22)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 5.00	-5.00, 15.00
Min, Max	-25.0, 75.0	-62.5, 35.0
Difference in change from baseline (95%CI)		1.99 (-9.25, 13.23)
P-value ^b		0.7234
Hedges'g (95% CI) ^c		0.10 (-0.46, 0.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	24	17
Mean (SD)	65.42 (29.30)	67.65 (24.25)
Median	72.50	75.00
25th, 75th Percentile	52.50, 87.50	55.00, 80.00
Min, Max	0.0, 100.0	15.0, 100.0
Week 26		
n	23	16
Mean (SD)	65.11 (30.89)	71.88 (16.32)
Median	80.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 90.00	60.00, 85.00
Min, Max	0.0, 100.0	50.0, 100.0
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	0.11 (16.22)	3.75 (24.19)
Median	0.00	2.50
25th, 75th Percentile	-10.00, 10.00	-10.00, 15.00
Min, Max	-50.0, 25.0	-30.0, 60.0
Week 52		
n	24	16
Mean (SD)	59.58 (29.82)	71.25 (14.78)
Median	65.00	75.00
25th, 75th Percentile	40.00, 82.50	60.00, 82.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.007.002_qs_sum_ovr_qol_care_fut_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	45.0, 90.0
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	-5.83 (20.36)	3.13 (18.15)
Median	-5.00	0.00
25th, 75th Percentile	-12.50, 0.00	-7.50, 5.00
Min, Max	-70.0, 40.0	-25.0, 45.0
Difference in change from baseline (95%CI)		8.96 (-3.79, 21.71)
P-value ^b		0.1632
Hedges'g (95% CI) ^c		0.45 (-0.19, 1.09)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	13	12
Mean (SD)	61.15 (29.80)	68.33 (22.50)
Median	60.00	67.50
25th, 75th Percentile	30.00, 90.00	52.50, 85.00
Min, Max	20.0, 100.0	35.0, 100.0
Week 26		
n	12	11
Mean (SD)	70.42 (22.10)	75.00 (18.17)
Median	77.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 85.00	60.00, 95.00
Min, Max	20.0, 100.0	40.0, 100.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	10.83 (17.56)	6.82 (23.48)
Median	17.50	0.00
25th, 75th Percentile	0.00, 22.50	-5.00, 20.00
Min, Max	-20.0, 35.0	-35.0, 50.0
Week 52		
n	12	10
Mean (SD)	71.25 (23.27)	75.50 (21.27)
Median	72.50	72.50
25th, 75th Percentile	50.00, 95.00	70.00, 95.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	35.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	12	10
Mean (SD)	7.50 (16.17)	5.50 (24.99)
Median	7.50	2.50
25th, 75th Percentile	0.00, 15.00	0.00, 10.00
Min, Max	-25.0, 45.0	-45.0, 50.0
Difference in change from baseline (95%CI)		-2.00 (-20.41, 16.41)
P-value ^b		0.8230
Hedges'g (95% CI) ^c		-0.09 (-0.93, 0.75)
P-value for interaction term, treatment * [Age at Baseline]		0.5462

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	47	46
Mean (SD)	69.57 (31.29)	68.42 (25.44)
Median	80.00	72.50
25th, 75th Percentile	55.00, 95.00	55.00, 90.00
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	46	43
Mean (SD)	70.24 (30.50)	73.95 (21.78)
Median	85.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 95.00	55.00, 95.00
Min, Max	0.0, 100.0	15.0, 100.0
Change from baseline to Week 26 ^a		
n	45	42
Mean (SD)	1.58 (20.83)	6.37 (22.31)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 10.00	-5.00, 20.00
Min, Max	-95.0, 40.0	-42.5, 60.0
Week 52		
n	46	47
Mean (SD)	69.67 (29.24)	72.77 (21.86)
Median	75.00	75.00
25th, 75th Percentile	55.00, 95.00	60.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	45	45
Mean (SD)	-1.00 (21.65)	3.28 (21.68)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 5.00	-5.00, 15.00
Min, Max	-70.0, 75.0	-62.5, 50.0
Difference in change from baseline (95%CI)		4.28 (-4.80, 13.35)
P-value ^b		0.3515
Hedges'g (95% CI) ^c		0.20 (-0.22, 0.61)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	13	12
Mean (SD)	63.08 (28.98)	72.92 (19.36)
Median	60.00	75.00
25th, 75th Percentile	35.00, 90.00	62.50, 85.00
Min, Max	15.0, 100.0	35.0, 100.0
Week 26		
n	13	11
Mean (SD)	64.71 (25.90)	74.09 (16.40)
Median	65.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 85.00	60.00, 95.00
Min, Max	5.0, 100.0	50.0, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	1.63 (15.56)	0.00 (17.46)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 15.00	-5.00, 5.00
Min, Max	-20.0, 30.0	-30.0, 40.0
Week 52		
n	13	10
Mean (SD)	63.85 (29.09)	80.00 (11.55)
Median	60.00	75.00
25th, 75th Percentile	45.00, 95.00	70.00, 85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	10.0, 100.0	70.0, 100.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	0.77 (8.86)	3.50 (12.48)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 5.00	-5.00, 5.00
Min, Max	-15.0, 15.0	-10.0, 35.0
Difference in change from baseline (95%CI)		2.73 (-6.51, 11.97)
P-value ^b		0.5456
Hedges'g (95% CI) ^c		0.25 (-0.58, 1.07)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.8698

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	10	15
Mean (SD)	71.50 (31.45)	65.00 (24.28)
Median	75.00	70.00
25th, 75th Percentile	60.00, 100.00	55.00, 80.00
Min, Max	0.0, 100.0	15.0, 100.0
Week 26		
n	10	12
Mean (SD)	61.13 (37.73)	76.25 (19.79)
Median	72.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 95.00	60.00, 95.00
Min, Max	0.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-10.38 (31.80)	11.25 (27.97)
Median	-2.50	2.50
25th, 75th Percentile	-15.00, 5.00	-5.00, 35.00
Min, Max	-95.0, 20.0	-30.0, 60.0
Week 52		
n	10	13
Mean (SD)	69.50 (30.13)	74.23 (16.69)
Median	72.50	75.00
25th, 75th Percentile	60.00, 95.00	60.00, 85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	45.0, 100.0
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	-2.00 (8.88)	9.23 (19.13)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 0.00	0.00, 15.00
Min, Max	-20.0, 10.0	-25.0, 45.0
Difference in change from baseline (95%CI)		11.23 (-1.39, 23.85)
P-value ^b		0.0779
Hedges'g (95% CI) ^c		0.69 (-0.16, 1.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	23	18
Mean (SD)	71.96 (29.76)	69.03 (24.84)
Median	85.00	72.50
25th, 75th Percentile	50.00, 95.00	55.00, 90.00
Min, Max	0.0, 100.0	15.0, 100.0
Week 26		
n	22	18
Mean (SD)	75.68 (25.32)	70.00 (21.00)
Median	85.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 95.00	55.00, 85.00
Min, Max	10.0, 100.0	20.0, 100.0
Change from baseline to Week 26 ^a		
n	21	18
Mean (SD)	5.71 (11.97)	0.97 (23.06)
Median	5.00	0.00
25th, 75th Percentile	0.00, 10.00	-5.00, 15.00
Min, Max	-15.0, 35.0	-42.5, 50.0
Week 52		
n	22	18
Mean (SD)	72.27 (27.24)	70.28 (24.40)
Median	77.50	75.00
25th, 75th Percentile	55.00, 95.00	65.00, 85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	5.0, 100.0	0.0, 95.0
Change from baseline to Week 52 ^a		
n	21	18
Mean (SD)	-2.14 (17.07)	1.25 (24.68)
Median	0.00	-2.50
25th, 75th Percentile	-15.00, 5.00	-5.00, 5.00
Min, Max	-30.0, 45.0	-62.5, 50.0
Difference in change from baseline (95%CI)		3.39 (-10.22, 17.00)
P-value ^b		0.6165
Hedges'g (95% CI) ^c		0.16 (-0.47, 0.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.007.005_qs_sum_ovr_qol_care_fut_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	19	20
Mean (SD)	59.47 (33.78)	70.75 (26.02)
Median	70.00	75.00
25th, 75th Percentile	25.00, 90.00	55.00, 90.00
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	19	19
Mean (SD)	61.91 (31.18)	75.53 (20.74)
Median	65.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.00, 90.00	65.00, 90.00
Min, Max	0.0, 100.0	15.0, 100.0
Change from baseline to Week 26 ^a		
n	19	18
Mean (SD)	2.43 (19.08)	6.67 (12.49)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 10.00	0.00, 15.00
Min, Max	-50.0, 40.0	-15.0, 35.0
Week 52		
n	19	21
Mean (SD)	60.53 (33.20)	76.90 (18.47)
Median	60.00	75.00
25th, 75th Percentile	35.00, 95.00	70.00, 95.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	19	19
Mean (SD)	1.05 (28.12)	2.89 (13.98)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-5.00, 10.00
Min, Max	-70.0, 75.0	-15.0, 35.0
Difference in change from baseline (95%CI)		1.84 (-12.95, 16.64)
P-value ^b		0.8001
Hedges'g (95% CI) ^c		0.08 (-0.56, 0.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	8	5
Mean (SD)	73.75 (25.32)	78.00 (16.81)
Median	80.00	75.00
25th, 75th Percentile	72.50, 87.50	65.00, 90.00
Min, Max	15.0, 95.0	60.0, 100.0
Week 26		
n	8	5
Mean (SD)	77.50 (21.71)	77.00 (25.64)
Median	85.00	95.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	70.00, 92.50	60.00, 95.00
Min, Max	30.0, 95.0	40.0, 95.0
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	3.75 (15.29)	-1.00 (25.10)
Median	5.00	-5.00
25th, 75th Percentile	-7.50, 15.00	-5.00, 5.00
Min, Max	-20.0, 25.0	-35.0, 35.0
Week 52		
n	8	5
Mean (SD)	75.00 (22.36)	75.00 (26.93)
Median	77.50	80.00
25th, 75th Percentile	65.00, 92.50	75.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	1.25 (9.91)	-3.00 (27.75)
Median	2.50	0.00
25th, 75th Percentile	-5.00, 7.50	-10.00, 10.00
Min, Max	-15.0, 15.0	-45.0, 30.0
Difference in change from baseline (95%CI)		-4.25 (-38.17, 29.67)
P-value ^b		0.7560
Hedges'g (95% CI) ^c		-0.21 (-1.33, 0.91)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.7188

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	19	19
Mean (SD)	74.21 (26.84)	73.29 (23.03)
Median	80.00	70.00
25th, 75th Percentile	55.00, 100.00	60.00, 100.00
Min, Max	15.0, 100.0	25.0, 100.0
Week 26		
n	19	15
Mean (SD)	75.07 (27.37)	70.00 (22.99)
Median	85.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 95.00	50.00, 90.00
Min, Max	5.0, 100.0	20.0, 100.0
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	0.86 (12.83)	-0.50 (27.09)
Median	0.00	0.00
25th, 75th Percentile	-10.00, 10.00	-30.00, 20.00
Min, Max	-18.8, 25.0	-42.5, 50.0
Week 52		
n	18	18
Mean (SD)	73.33 (29.85)	73.06 (25.04)
Median	80.00	77.50
25th, 75th Percentile	60.00, 100.00	65.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	10.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	-0.28 (13.34)	-0.42 (25.59)
Median	0.00	2.50
25th, 75th Percentile	-5.00, 5.00	-10.00, 10.00
Min, Max	-20.0, 40.0	-62.5, 50.0
Difference in change from baseline (95%CI)		-0.14 (-14.13, 13.85)
P-value ^b		0.9839
Hedges'g (95% CI) ^c		-0.01 (-0.66, 0.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	18	14
Mean (SD)	58.61 (32.67)	67.14 (33.95)
Median	70.00	82.50
25th, 75th Percentile	30.00, 90.00	35.00, 95.00
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	16	14
Mean (SD)	63.13 (30.71)	74.29 (24.95)
Median	65.00	82.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 90.00	55.00, 95.00
Min, Max	0.0, 100.0	15.0, 100.0
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	6.88 (21.59)	7.14 (20.16)
Median	10.00	2.50
25th, 75th Percentile	0.00, 22.50	0.00, 5.00
Min, Max	-50.0, 35.0	-25.0, 60.0
Week 52		
n	17	14
Mean (SD)	59.71 (32.04)	72.50 (18.48)
Median	65.00	70.00
25th, 75th Percentile	45.00, 85.00	60.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	35.0, 100.0
Change from baseline to Week 52 ^a		
n	17	14
Mean (SD)	-0.59 (23.78)	5.36 (20.71)
Median	0.00	-2.50
25th, 75th Percentile	-5.00, 10.00	-10.00, 25.00
Min, Max	-70.0, 45.0	-15.0, 45.0
Difference in change from baseline (95%CI)		5.95 (-10.63, 22.52)
P-value ^b		0.4690
Hedges'g (95% CI) ^c		0.26 (-0.45, 0.97)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	23	25
Mean (SD)	70.65 (31.60)	67.60 (18.77)
Median	80.00	70.00
25th, 75th Percentile	60.00, 95.00	60.00, 80.00
Min, Max	0.0, 100.0	15.0, 100.0
Week 26		
n	24	25
Mean (SD)	68.18 (30.44)	76.20 (16.79)
Median	82.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.63, 90.00	65.00, 95.00
Min, Max	0.0, 100.0	45.0, 100.0
Change from baseline to Week 26 ^a		
n	23	24
Mean (SD)	-1.47 (22.73)	7.29 (18.18)
Median	0.00	2.50
25th, 75th Percentile	-5.00, 5.00	-5.00, 15.00
Min, Max	-95.0, 40.0	-30.0, 45.0
Week 52		
n	24	25
Mean (SD)	70.83 (25.99)	75.60 (18.73)
Median	77.50	75.00
25th, 75th Percentile	52.50, 92.50	70.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	25.0, 100.0
Change from baseline to Week 52 ^a		
n	23	23
Mean (SD)	-0.87 (20.82)	5.00 (15.08)
Median	0.00	5.00
25th, 75th Percentile	-10.00, 10.00	-5.00, 10.00
Min, Max	-30.0, 75.0	-25.0, 35.0
Difference in change from baseline (95%CI)		5.87 (-4.93, 16.67)
P-value ^b		0.2794
Hedges'g (95% CI) ^c		0.32 (-0.27, 0.90)
P-value for interaction term, treatment * [Baseline AGV]		0.7593

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.007.006_qs_sum_ovr_qol_care_fut_bhagv_301_fas.pdf+rff

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.7.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	40	44
Mean (SD)	66.38 (30.53)	70.57 (23.73)
Median	70.00	75.00
25th, 75th Percentile	50.00, 92.50	57.50, 90.00
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	40	41
Mean (SD)	67.66 (30.15)	76.10 (18.73)
Median	80.00	80.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.00, 92.50	65.00, 95.00
Min, Max	0.0, 100.0	15.0, 100.0
Change from baseline to Week 26 ^a		
n	39	41
Mean (SD)	2.21 (22.70)	6.10 (19.83)
Median	0.00	5.00
25th, 75th Percentile	-5.00, 15.00	-5.00, 15.00
Min, Max	-95.0, 40.0	-35.0, 60.0
Week 52		
n	39	43
Mean (SD)	69.23 (28.13)	76.98 (16.19)
Median	75.00	75.00
25th, 75th Percentile	55.00, 95.00	70.00, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	30.0, 100.0
Change from baseline to Week 52 ^a		
n	38	42
Mean (SD)	1.71 (22.61)	5.36 (18.19)
Median	0.00	2.50
25th, 75th Percentile	-5.00, 10.00	-5.00, 15.00
Min, Max	-70.0, 75.0	-45.0, 50.0
Difference in change from baseline (95%CI)		3.65 (-5.45, 12.74)
P-value ^b		0.4272
Hedges'g (95% CI) ^c		0.18 (-0.26, 0.62)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.7.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Future Score		
Baseline		
n	20	14
Mean (SD)	71.75 (31.47)	65.54 (26.24)
Median	80.00	72.50
25th, 75th Percentile	65.00, 95.00	55.00, 80.00
Min, Max	0.0, 100.0	15.0, 100.0
Week 26		
n	19	13
Mean (SD)	71.91 (28.40)	67.31 (25.55)
Median	85.00	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 90.00	50.00, 95.00
Min, Max	0.0, 100.0	20.0, 100.0
Change from baseline to Week 26 ^a		
n	19	12
Mean (SD)	0.33 (11.46)	1.46 (26.77)
Median	0.00	0.00
25th, 75th Percentile	-5.00, 5.00	-15.00, 20.00
Min, Max	-20.0, 30.0	-42.5, 45.0
Week 52		
n	20	14
Mean (SD)	66.75 (31.47)	65.00 (29.22)
Median	65.00	67.50
25th, 75th Percentile	50.00, 95.00	50.00, 85.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.7.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Future Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	20	13
Mean (SD)	-5.00 (10.51)	-3.27 (25.44)
Median	-5.00	0.00
25th, 75th Percentile	-10.00, 0.00	-15.00, 5.00
Min, Max	-25.0, 10.0	-62.5, 35.0
Difference in change from baseline (95%CI)		1.73 (-14.15, 17.61)
P-value ^b		0.8192
Hedges'g (95% CI) ^c		0.09 (-0.60, 0.79)
P-value for interaction term, treatment * [Ethnicity]		0.8178

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	33	31
Mean (SD)	64.55 (24.46)	58.95 (21.31)
Median	72.50	60.00
25th, 75th Percentile	50.00, 82.50	45.00, 77.50
Min, Max	15.0, 100.0	10.0, 97.5
Week 26		
n	31	28
Mean (SD)	68.95 (21.67)	60.74 (22.25)
Median	72.50	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	55.00, 90.00	52.50, 78.75
Min, Max	22.5, 95.0	0.0, 90.0
Change from baseline to Week 26 ^a		
n	31	28
Mean (SD)	4.76 (21.92)	2.44 (17.76)
Median	2.50	3.75
25th, 75th Percentile	-5.00, 20.00	-5.00, 10.42
Min, Max	-60.0, 65.0	-57.5, 32.5
Week 52		
n	31	30
Mean (SD)	67.97 (21.95)	56.75 (24.57)
Median	70.00	55.00
25th, 75th Percentile	52.50, 85.00	42.50, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	17.5, 97.5	0.0, 97.5
Change from baseline to Week 52 ^a		
n	31	30
Mean (SD)	2.00 (21.78)	-2.25 (20.26)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 10.00	-12.50, 12.50
Min, Max	-30.0, 72.5	-60.0, 35.0
Difference in change from baseline (95%CI)		-4.25 (-15.03, 6.54)
P-value ^b		0.4338
Hedges'g (95% CI) ^c		-0.20 (-0.70, 0.30)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	28	29
Mean (SD)	50.54 (23.37)	58.93 (20.65)
Median	48.75	62.50
25th, 75th Percentile	35.00, 66.25	47.50, 72.50
Min, Max	0.0, 100.0	12.5, 100.0
Week 26		
n	28	27
Mean (SD)	56.16 (23.43)	63.06 (19.33)
Median	65.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.8.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	41.25, 70.00	52.50, 77.50
Min, Max	0.0, 92.5	17.5, 95.0
Change from baseline to Week 26 ^a		
n	28	27
Mean (SD)	5.63 (12.37)	3.19 (13.36)
Median	6.25	2.50
25th, 75th Percentile	-5.00, 17.50	-5.00, 10.00
Min, Max	-20.0, 25.0	-16.4, 40.0
Week 52		
n	28	27
Mean (SD)	57.23 (21.93)	60.29 (19.88)
Median	65.00	67.50
25th, 75th Percentile	45.00, 73.75	45.00, 77.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.1
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 92.5	10.0, 90.0
Change from baseline to Week 52 ^a		
n	28	27
Mean (SD)	6.70 (14.72)	0.05 (16.49)
Median	3.75	-2.50
25th, 75th Percentile	-5.00, 20.00	-10.00, 10.00
Min, Max	-20.0, 37.5	-42.5, 55.0
Difference in change from baseline (95%CI)		-6.64 (-15.09, 1.80)
P-value ^b		0.1205
Hedges'g (95% CI) ^c		-0.42 (-0.95, 0.12)
P-value for interaction term, treatment * [Sex]		0.7307

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	24	31
Mean (SD)	59.48 (21.45)	58.27 (21.02)
Median	65.00	60.00
25th, 75th Percentile	45.00, 77.50	42.50, 72.50
Min, Max	15.0, 100.0	10.0, 97.5
Week 26		
n	24	28
Mean (SD)	61.56 (19.57)	57.68 (23.70)
Median	65.00	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 72.50	50.00, 76.25
Min, Max	22.5, 92.5	0.0, 87.5
Change from baseline to Week 26 ^a		
n	24	28
Mean (SD)	2.08 (17.76)	-0.76 (15.93)
Median	2.50	0.00
25th, 75th Percentile	-6.25, 15.00	-10.00, 7.50
Min, Max	-60.0, 22.5	-57.5, 32.5
Week 52		
n	23	31
Mean (SD)	60.98 (19.61)	54.11 (23.14)
Median	62.50	55.00
25th, 75th Percentile	47.50, 75.00	40.00, 70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	17.5, 92.5	0.0, 97.5
Change from baseline to Week 52 ^a		
n	23	31
Mean (SD)	1.09 (16.87)	-4.16 (17.39)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 12.50	-10.00, 10.00
Min, Max	-30.0, 32.5	-60.0, 35.0
Difference in change from baseline (95%CI)		-5.24 (-14.73, 4.24)
P-value ^b		0.2722
Hedges'g (95% CI) ^c		-0.30 (-0.84, 0.24)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	24	17
Mean (SD)	54.27 (28.71)	52.79 (18.30)
Median	60.00	57.50
25th, 75th Percentile	27.50, 80.00	42.50, 70.00
Min, Max	0.0, 95.0	22.5, 77.5
Week 26		
n	23	16
Mean (SD)	60.33 (27.73)	60.21 (16.23)
Median	70.00	59.17

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.001.008.002_qs_sum_ovr_qol_care_eff_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.00, 82.50	46.25, 77.50
Min, Max	0.0, 90.0	35.0, 82.5
Change from baseline to Week 26 ^a		
n	23	16
Mean (SD)	6.20 (21.06)	7.86 (12.85)
Median	2.50	6.25
25th, 75th Percentile	-5.00, 20.00	2.50, 10.42
Min, Max	-37.5, 65.0	-12.5, 40.0
Week 52		
n	24	16
Mean (SD)	58.94 (26.15)	57.05 (19.37)
Median	67.50	62.50
25th, 75th Percentile	37.50, 80.00	40.00, 73.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 97.5	22.5, 82.5
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	4.66 (21.25)	4.71 (17.59)
Median	0.00	1.25
25th, 75th Percentile	-8.75, 8.75	-6.25, 13.75
Min, Max	-20.0, 72.5	-17.5, 55.0
Difference in change from baseline (95%CI)		0.04 (-12.95, 13.03)
P-value ^b		0.9950
Hedges'g (95% CI) ^c		0.00 (-0.63, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	13	12
Mean (SD)	62.69 (23.53)	69.38 (21.27)
Median	57.50	71.25
25th, 75th Percentile	45.00, 82.50	58.75, 83.75
Min, Max	30.0, 100.0	22.5, 100.0
Week 26		
n	12	11
Mean (SD)	70.42 (20.69)	75.00 (12.70)
Median	67.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.75, 92.50	65.00, 85.00
Min, Max	30.0, 95.0	55.0, 95.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	9.38 (9.89)	4.55 (17.42)
Median	10.00	-2.50
25th, 75th Percentile	-1.25, 18.75	-5.00, 12.50
Min, Max	-5.0, 22.5	-25.0, 37.5
Week 52		
n	12	10
Mean (SD)	74.38 (16.24)	74.00 (18.97)
Median	75.00	77.50
25th, 75th Percentile	61.25, 86.25	67.50, 90.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.2
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	50.0, 97.5	42.5, 95.0
Change from baseline to Week 52 ^a		
n	12	10
Mean (SD)	9.38 (17.09)	-1.25 (22.37)
Median	13.75	-2.50
25th, 75th Percentile	0.00, 18.75	-10.00, 17.50
Min, Max	-30.0, 37.5	-47.5, 35.0
Difference in change from baseline (95%CI)		-10.63 (-28.17, 6.92)
P-value ^b		0.2210
Hedges'g (95% CI) ^c		-0.52 (-1.37, 0.34)
P-value for interaction term, treatment * [Age at Baseline]		0.5550

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	48	48
Mean (SD)	56.46 (24.89)	56.23 (20.41)
Median	57.50	57.50
25th, 75th Percentile	37.50, 77.50	42.50, 71.25
Min, Max	0.0, 100.0	10.0, 97.5
Week 26		
n	46	44
Mean (SD)	61.03 (22.81)	58.20 (20.95)
Median	65.00	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 80.00	50.00, 76.25
Min, Max	0.0, 92.5	0.0, 87.5
Change from baseline to Week 26 ^a		
n	46	44
Mean (SD)	5.16 (19.32)	2.03 (16.36)
Median	3.75	2.50
25th, 75th Percentile	-5.00, 17.50	-8.75, 10.00
Min, Max	-60.0, 65.0	-57.5, 40.0
Week 52		
n	46	47
Mean (SD)	59.88 (22.57)	54.42 (22.22)
Median	62.50	55.00
25th, 75th Percentile	45.00, 75.00	40.00, 70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 97.5	0.0, 97.5
Change from baseline to Week 52 ^a		
n	46	47
Mean (SD)	2.81 (19.82)	-1.78 (20.01)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 12.50	-12.50, 10.00
Min, Max	-30.0, 72.5	-60.0, 55.0
Difference in change from baseline (95%CI)		-4.59 (-12.80, 3.61)
P-value ^b		0.2691
Hedges'g (95% CI) ^c		-0.23 (-0.64, 0.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	13	12
Mean (SD)	64.23 (24.35)	69.79 (19.58)
Median	67.50	71.25
25th, 75th Percentile	47.50, 80.00	61.25, 77.50
Min, Max	20.0, 100.0	22.5, 100.0
Week 26		
n	13	11
Mean (SD)	69.42 (24.48)	76.59 (11.47)
Median	70.00	77.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.00, 92.50	65.00, 85.00
Min, Max	12.5, 95.0	60.0, 95.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	5.19 (12.10)	5.91 (12.36)
Median	7.50	5.00
25th, 75th Percentile	-2.50, 15.00	-5.00, 12.50
Min, Max	-20.0, 20.0	-5.0, 37.5
Week 52		
n	13	10
Mean (SD)	73.46 (19.00)	77.25 (10.24)
Median	75.00	77.50
25th, 75th Percentile	67.50, 85.00	70.00, 82.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.3
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 97.5	62.5, 95.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	9.23 (13.90)	1.75 (7.64)
Median	5.00	0.00
25th, 75th Percentile	-2.50, 17.50	-2.50, 5.00
Min, Max	-7.5, 37.5	-10.0, 17.5
Difference in change from baseline (95%CI)		-7.48 (-17.66, 2.70)
P-value ^b		0.1413
Hedges'g (95% CI) ^c		-0.62 (-1.46, 0.23)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.7418

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	10	15
Mean (SD)	50.00 (31.99)	57.09 (17.19)
Median	46.25	57.50
25th, 75th Percentile	25.00, 82.50	42.50, 72.50
Min, Max	0.0, 100.0	22.5, 88.9
Week 26		
n	10	12
Mean (SD)	52.50 (29.79)	65.83 (17.66)
Median	62.50	71.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 70.00	56.25, 80.00
Min, Max	0.0, 92.5	30.0, 85.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	2.50 (12.42)	6.97 (14.84)
Median	-2.50	8.75
25th, 75th Percentile	-7.50, 17.50	-3.75, 13.75
Min, Max	-12.5, 20.0	-16.4, 40.0
Week 52		
n	10	13
Mean (SD)	54.50 (31.51)	63.29 (19.28)
Median	62.50	67.50
25th, 75th Percentile	30.00, 72.50	47.50, 77.78

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 95.0	35.0, 92.5
Change from baseline to Week 52 ^a		
n	10	13
Mean (SD)	4.50 (17.07)	6.45 (18.08)
Median	5.00	5.00
25th, 75th Percentile	-7.50, 20.00	-6.39, 10.00
Min, Max	-25.0, 27.5	-17.5, 55.0
Difference in change from baseline (95%CI)		1.95 (-13.49, 17.39)
P-value ^b		0.7951
Hedges'g (95% CI) ^c		0.11 (-0.72, 0.93)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	24	18
Mean (SD)	60.94 (24.59)	55.00 (17.95)
Median	61.25	55.00
25th, 75th Percentile	43.75, 78.75	47.50, 70.00
Min, Max	15.0, 100.0	12.5, 80.0
Week 26		
n	22	18
Mean (SD)	68.30 (17.36)	55.88 (20.82)
Median	66.25	59.17

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 82.50	50.00, 77.50
Min, Max	30.0, 95.0	7.5, 80.0
Change from baseline to Week 26 ^a		
n	22	18
Mean (SD)	8.18 (14.38)	0.88 (19.04)
Median	11.25	3.75
25th, 75th Percentile	-5.00, 17.50	-7.50, 10.00
Min, Max	-15.0, 42.5	-57.5, 32.5
Week 52		
n	22	18
Mean (SD)	65.80 (21.98)	52.64 (21.41)
Median	67.50	56.25
25th, 75th Percentile	52.50, 85.00	50.00, 67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	17.5, 97.5	5.0, 85.0
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	3.18 (17.55)	-2.36 (19.03)
Median	2.50	-2.50
25th, 75th Percentile	-10.00, 15.00	-10.00, 2.50
Min, Max	-30.0, 50.0	-60.0, 35.0
Difference in change from baseline (95%CI)		-5.54 (-17.27, 6.18)
P-value ^b		0.3446
Hedges'g (95% CI) ^c		-0.30 (-0.92, 0.33)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	19	22
Mean (SD)	56.45 (21.58)	59.32 (24.99)
Median	62.50	62.50
25th, 75th Percentile	32.50, 75.00	40.00, 77.50
Min, Max	20.0, 87.5	10.0, 100.0
Week 26		
n	19	20
Mean (SD)	56.97 (23.71)	62.38 (23.19)
Median	67.50	66.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 72.50	52.50, 80.00
Min, Max	12.5, 90.0	0.0, 95.0
Change from baseline to Week 26 ^a		
n	19	20
Mean (SD)	0.53 (25.13)	4.00 (12.78)
Median	0.00	1.25
25th, 75th Percentile	-7.50, 10.00	-5.00, 6.25
Min, Max	-60.0, 65.0	-12.5, 37.5
Week 52		
n	19	21
Mean (SD)	61.18 (18.90)	57.26 (24.62)
Median	65.00	55.00
25th, 75th Percentile	45.00, 75.00	40.00, 77.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 97.5	0.0, 97.5
Change from baseline to Week 52 ^a		
n	19	21
Mean (SD)	4.74 (23.00)	-3.81 (16.41)
Median	0.00	-5.00
25th, 75th Percentile	-7.50, 20.00	-12.50, 2.50
Min, Max	-30.0, 72.5	-42.5, 35.0
Difference in change from baseline (95%CI)		-8.55 (-21.24, 4.15)
P-value ^b		0.1810
Hedges'g (95% CI) ^c		-0.42 (-1.05, 0.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	8	5
Mean (SD)	63.75 (24.49)	77.00 (14.73)
Median	75.00	72.50
25th, 75th Percentile	41.25, 82.50	65.00, 90.00
Min, Max	25.0, 87.5	62.5, 95.0
Week 26		
n	8	5
Mean (SD)	75.00 (21.75)	72.00 (14.08)
Median	83.75	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 91.25	65.00, 77.50
Min, Max	32.5, 92.5	52.5, 90.0
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	11.25 (8.35)	-5.00 (14.68)
Median	8.75	-5.00
25th, 75th Percentile	3.75, 20.00	-12.50, 5.00
Min, Max	2.5, 22.5	-25.0, 12.5
Week 52		
n	8	5
Mean (SD)	69.31 (18.31)	71.50 (21.11)
Median	76.25	80.00
25th, 75th Percentile	58.47, 82.50	57.50, 82.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.5
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	35.0, 85.0	42.5, 95.0
Change from baseline to Week 52 ^a		
n	8	5
Mean (SD)	5.56 (15.83)	-5.50 (25.34)
Median	2.50	0.00
25th, 75th Percentile	-6.25, 16.25	-7.50, 10.00
Min, Max	-13.1, 32.5	-47.5, 17.5
Difference in change from baseline (95%CI)		-11.06 (-35.93, 13.82)
P-value ^b		0.3490
Hedges'g (95% CI) ^c		-0.52 (-1.65, 0.63)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.7044

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	19	19
Mean (SD)	62.37 (26.78)	58.89 (20.33)
Median	65.00	60.00
25th, 75th Percentile	45.00, 85.00	42.50, 72.50
Min, Max	15.0, 100.0	25.0, 97.5
Week 26		
n	19	15
Mean (SD)	65.66 (25.93)	55.67 (18.62)
Median	72.50	57.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	52.50, 85.00	52.50, 67.50
Min, Max	10.0, 95.0	7.5, 80.0
Change from baseline to Week 26 ^a		
n	19	15
Mean (SD)	3.29 (13.12)	-3.76 (21.10)
Median	2.50	-2.50
25th, 75th Percentile	-7.50, 17.50	-12.50, 10.00
Min, Max	-20.0, 22.5	-57.5, 27.5
Week 52		
n	18	18
Mean (SD)	65.11 (27.28)	56.11 (21.71)
Median	72.22	53.75
25th, 75th Percentile	37.50, 85.00	42.50, 70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	15.0, 97.5	5.0, 97.5
Change from baseline to Week 52 ^a		
n	18	18
Mean (SD)	2.05 (12.56)	-2.86 (24.83)
Median	1.25	0.00
25th, 75th Percentile	-7.50, 12.50	-17.50, 12.50
Min, Max	-25.0, 20.0	-60.0, 35.0
Difference in change from baseline (95%CI)		-4.91 (-18.41, 8.60)
P-value ^b		0.4613
Hedges'g (95% CI) ^c		-0.24 (-0.90, 0.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	18	14
Mean (SD)	61.25 (23.00)	54.29 (25.58)
Median	65.00	56.25
25th, 75th Percentile	42.50, 80.00	35.00, 72.50
Min, Max	20.0, 100.0	10.0, 100.0
Week 26		
n	16	14
Mean (SD)	68.59 (21.95)	60.77 (24.24)
Median	67.50	65.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	51.25, 90.00	50.00, 77.50
Min, Max	25.0, 95.0	0.0, 95.0
Change from baseline to Week 26 ^a		
n	16	14
Mean (SD)	8.44 (21.13)	6.49 (11.53)
Median	8.75	6.25
25th, 75th Percentile	-1.25, 17.50	0.00, 10.00
Min, Max	-37.5, 65.0	-10.0, 40.0
Week 52		
n	17	14
Mean (SD)	71.18 (18.03)	59.48 (27.21)
Median	75.00	63.75
25th, 75th Percentile	55.00, 85.00	50.00, 77.78

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	42.5, 97.5	0.0, 92.5
Change from baseline to Week 52 ^a		
n	17	14
Mean (SD)	8.38 (23.13)	5.20 (18.11)
Median	5.00	0.00
25th, 75th Percentile	-2.50, 15.00	-10.00, 15.00
Min, Max	-30.0, 72.5	-15.0, 55.0
Difference in change from baseline (95%CI)		-3.18 (-18.71, 12.34)
P-value ^b		0.6779
Hedges'g (95% CI) ^c		-0.15 (-0.85, 0.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	24	27
Mean (SD)	52.40 (24.37)	61.39 (18.76)
Median	53.75	62.50
25th, 75th Percentile	31.25, 75.00	52.50, 72.50
Min, Max	0.0, 87.5	12.5, 95.0
Week 26		
n	24	26
Mean (SD)	56.88 (21.32)	66.06 (19.61)
Median	62.50	73.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 71.25	52.50, 82.50
Min, Max	0.0, 90.0	17.5, 90.0
Change from baseline to Week 26 ^a		
n	24	26
Mean (SD)	4.48 (19.24)	4.62 (13.09)
Median	5.00	3.75
25th, 75th Percentile	-5.00, 17.50	-5.00, 12.50
Min, Max	-60.0, 42.5	-12.5, 37.5
Week 52		
n	24	25
Mean (SD)	55.31 (19.38)	59.50 (20.59)
Median	56.25	57.50
25th, 75th Percentile	46.25, 68.75	42.50, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.6
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 87.5	10.0, 95.0
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	2.92 (19.50)	-3.50 (12.16)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 21.25	-10.00, 2.50
Min, Max	-30.0, 50.0	-42.5, 17.5
Difference in change from baseline (95%CI)		-6.42 (-15.86, 3.02)
P-value ^b		0.1770
Hedges'g (95% CI) ^c		-0.39 (-0.95, 0.18)
P-value for interaction term, treatment * [Baseline AGV]		0.9311

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.1.8.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	41	45
Mean (SD)	57.44 (26.68)	59.20 (20.91)
Median	62.50	62.50
25th, 75th Percentile	32.50, 77.50	45.00, 72.50
Min, Max	0.0, 100.0	10.0, 100.0
Week 26		
n	40	42
Mean (SD)	60.50 (25.52)	63.29 (18.88)
Median	67.50	63.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 80.00	52.50, 77.50
Min, Max	0.0, 95.0	0.0, 95.0
Change from baseline to Week 26 ^a		
n	40	42
Mean (SD)	3.69 (19.46)	2.96 (14.07)
Median	3.75	1.25
25th, 75th Percentile	-5.00, 17.50	-7.50, 10.00
Min, Max	-60.0, 65.0	-25.0, 40.0
Week 52		
n	39	43
Mean (SD)	60.38 (25.06)	60.58 (20.34)
Median	67.50	57.50
25th, 75th Percentile	45.00, 82.50	47.50, 77.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	0.0, 97.5	0.0, 95.0
Change from baseline to Week 52 ^a		
n	39	43
Mean (SD)	2.18 (18.93)	0.49 (17.34)
Median	0.00	-2.50
25th, 75th Percentile	-10.00, 15.00	-10.00, 10.00
Min, Max	-30.0, 72.5	-47.5, 55.0
Difference in change from baseline (95%CI)		-1.69 (-9.66, 6.28)
P-value ^b		0.6744
Hedges'g (95% CI) ^c		-0.09 (-0.53, 0.34)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Caregiver-Reported QoLISSY : Effects Score		
Baseline		
n	20	15
Mean (SD)	59.50 (20.94)	58.17 (21.22)
Median	60.00	60.00
25th, 75th Percentile	45.00, 77.50	47.50, 72.50
Min, Max	20.0, 95.0	12.5, 97.5
Week 26		
n	19	13
Mean (SD)	67.89 (17.02)	57.31 (26.11)
Median	67.50	70.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.1.8.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.00, 82.50	37.50, 77.50
Min, Max	37.5, 92.5	7.5, 87.5
Change from baseline to Week 26 ^a		
n	19	13
Mean (SD)	8.29 (13.97)	2.31 (20.50)
Median	7.50	5.00
25th, 75th Percentile	-2.50, 20.00	0.00, 10.00
Min, Max	-15.0, 42.5	-57.5, 32.5
Week 52		
n	20	14
Mean (SD)	67.72 (15.47)	51.81 (27.41)
Median	68.47	52.50
25th, 75th Percentile	58.75, 80.00	30.00, 75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.1.8.7
Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Effects Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	37.5, 95.0	5.0, 97.5
Change from baseline to Week 52 ^a		
n	20	14
Mean (SD)	8.22 (18.23)	-6.23 (21.39)
Median	3.75	-1.25
25th, 75th Percentile	-5.00, 20.00	-10.00, 10.00
Min, Max	-20.0, 50.0	-60.0, 17.8
Difference in change from baseline (95%CI)		-14.45 (-28.35, -0.56)
P-value ^b		0.0420
Hedges'g (95% CI) ^c		-0.72 (-1.42, -0.01)
P-value for interaction term, treatment * [Ethnicity]		0.0981

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported QoLISSY : Total Score		
Baseline		
n	20	16
Mean (SD)	68.55 (15.51)	61.39 (19.86)
Median	67.02	65.45
25th, 75th Percentile	58.69, 81.95	46.70, 76.12
Min, Max	36.1, 90.6	20.5, 88.9
Week 26		
n	19	16
Mean (SD)	66.01 (16.34)	64.37 (18.59)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.28	67.17
25th, 75th Percentile	55.56, 80.56	44.62, 77.44
Min, Max	24.0, 92.0	33.3, 95.1
Change from baseline to Week 26 ^a		
n	17	15
Mean (SD)	-2.76 (19.59)	5.56 (13.83)
Median	-3.47	3.47
25th, 75th Percentile	-7.63, 6.25	-3.82, 21.18
Min, Max	-66.3, 26.8	-16.7, 28.8
Week 52		
n	24	18
Mean (SD)	65.42 (22.29)	61.73 (15.24)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.06	58.16
25th, 75th Percentile	51.57, 82.82	48.96, 74.31
Min, Max	9.0, 91.3	39.2, 90.6
Change from baseline to Week 52 ^a		
n	19	14
Mean (SD)	-1.90 (21.01)	3.79 (16.59)
Median	-1.04	0.53
25th, 75th Percentile	-10.21, 12.84	-8.68, 7.29
Min, Max	-63.9, 28.5	-15.8, 41.0
Difference in change from baseline (95%CI)		5.70 (-8.15, 19.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.4081
Hedges'g (95% CI) ^c		0.29 (-0.41, 0.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported QoLISSY : Total Score		
Baseline		
n	16	14
Mean (SD)	63.72 (16.81)	68.26 (14.39)
Median	66.33	68.75
25th, 75th Percentile	52.09, 75.65	59.38, 80.21
Min, Max	21.2, 85.1	37.5, 92.0
Week 26		
n	20	16
Mean (SD)	67.77 (16.22)	70.77 (12.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.58	71.53
25th, 75th Percentile	56.77, 77.39	66.15, 77.71
Min, Max	34.4, 93.1	35.8, 89.4
Change from baseline to Week 26 ^a		
n	16	11
Mean (SD)	4.20 (12.68)	3.22 (17.45)
Median	6.18	2.63
25th, 75th Percentile	-3.18, 11.51	-6.95, 15.28
Min, Max	-29.1, 29.9	-32.3, 29.0
Week 52		
n	20	18
Mean (SD)	63.79 (15.05)	73.05 (15.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	63.72	79.35
25th, 75th Percentile	52.26, 76.05	67.02, 83.96
Min, Max	31.9, 91.0	26.0, 91.3
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	0.33 (16.96)	4.98 (12.10)
Median	2.08	0.69
25th, 75th Percentile	-7.12, 9.21	-2.43, 9.21
Min, Max	-35.8, 39.2	-11.1, 30.9
Difference in change from baseline (95%CI)		4.65 (-7.20, 16.50)
P-value ^b		0.4271

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.2.1.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.30 (-0.46, 1.05)
P-value for interaction term, treatment * [Sex]		0.9090

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported QoLISSY : Total Score		
Baseline		
n	0	2
Mean (SD)		45.84 (19.16)
Median		45.84
25th, 75th Percentile		32.29, 59.38
Min, Max		32.3, 59.4
Week 26		
n	5	7
Mean (SD)	63.27 (16.52)	58.53 (19.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	59.03	71.53
25th, 75th Percentile	56.60, 75.35	35.77, 73.61
Min, Max	41.7, 83.7	33.3, 79.2
Change from baseline to Week 26 ^a		
n	0	1
Mean (SD)		1.04 (NA)
Median		1.04
25th, 75th Percentile		1.04, 1.04
Min, Max		1.0, 1.0
Week 52		
n	8	11
Mean (SD)	58.85 (26.59)	61.99 (19.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	60.07	67.02
25th, 75th Percentile	46.36, 78.82	48.61, 79.17
Min, Max	9.0, 91.3	26.0, 86.1
Change from baseline to Week 52 ^a		
n	0	2
Mean (SD)		13.20 (4.42)
Median		13.20
25th, 75th Percentile		10.07, 16.32
Min, Max		10.1, 16.3
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported QoLISSY : Total Score		
Baseline		
n	23	16
Mean (SD)	63.76 (16.70)	64.16 (17.26)
Median	65.63	65.46
25th, 75th Percentile	53.13, 74.56	56.25, 76.74
Min, Max	21.2, 90.3	20.5, 88.9
Week 26		
n	23	14
Mean (SD)	65.38 (18.33)	69.16 (16.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.28	70.84
25th, 75th Percentile	54.38, 78.82	64.93, 76.74
Min, Max	24.0, 93.1	41.7, 95.1
Change from baseline to Week 26 ^a		
n	22	14
Mean (SD)	0.58 (19.71)	5.19 (13.99)
Median	5.32	4.86
25th, 75th Percentile	-4.92, 9.82	-6.25, 15.28
Min, Max	-66.3, 29.9	-16.7, 29.0
Week 52		
n	23	16
Mean (SD)	61.66 (17.87)	68.76 (15.25)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	64.24	69.62
25th, 75th Percentile	50.70, 76.74	52.95, 81.60
Min, Max	26.4, 91.0	49.0, 91.3
Change from baseline to Week 52 ^a		
n	22	15
Mean (SD)	-2.91 (21.38)	4.59 (14.00)
Median	-3.30	1.04
25th, 75th Percentile	-13.54, 9.38	-3.47, 7.29
Min, Max	-63.9, 39.2	-12.2, 41.0
Difference in change from baseline (95%CI)		7.50 (-5.27, 20.26)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2411
Hedges'g (95% CI) ^c		0.39 (-0.27, 1.05)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported QoLISSY : Total Score		
Baseline		
n	13	12
Mean (SD)	71.08 (14.25)	68.30 (17.19)
Median	75.70	71.36
25th, 75th Percentile	61.46, 80.21	53.30, 81.78
Min, Max	41.7, 90.6	37.5, 92.0
Week 26		
n	11	11
Mean (SD)	71.77 (9.87)	71.30 (11.00)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.27	67.71
25th, 75th Percentile	64.58, 76.64	62.50, 80.56
Min, Max	55.2, 87.9	55.6, 90.3
Change from baseline to Week 26 ^a		
n	11	11
Mean (SD)	0.68 (8.92)	4.11 (17.86)
Median	0.35	2.63
25th, 75th Percentile	-6.94, 9.02	-6.94, 22.92
Min, Max	-11.1, 14.2	-32.3, 28.8
Week 52		
n	13	9
Mean (SD)	73.61 (13.73)	71.55 (14.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	79.17	78.47
25th, 75th Percentile	65.97, 83.33	61.81, 81.60
Min, Max	41.0, 88.9	46.5, 87.9
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	2.54 (14.33)	1.95 (16.68)
Median	2.78	-4.17
25th, 75th Percentile	-1.04, 6.25	-9.03, 5.91
Min, Max	-35.8, 25.7	-15.8, 33.6
Difference in change from baseline (95%CI)		-0.59 (-14.44, 13.26)
P-value ^b		0.9305

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.04 (-0.89, 0.81)
P-value for interaction term, treatment * [Age at Baseline]		0.4014

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported QoLISSY : Total Score		
Baseline		
n	23	18
Mean (SD)	67.05 (13.39)	58.87 (17.23)
Median	65.63	60.77
25th, 75th Percentile	58.34, 76.88	47.22, 71.18
Min, Max	36.1, 90.3	20.5, 88.9
Week 26		
n	26	21
Mean (SD)	65.21 (16.94)	63.73 (17.34)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	63.00	67.36
25th, 75th Percentile	55.21, 76.64	44.79, 73.61
Min, Max	24.0, 93.1	33.3, 95.1
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	-2.29 (19.01)	7.24 (14.36)
Median	-2.38	6.25
25th, 75th Percentile	-5.91, 7.12	-3.82, 21.18
Min, Max	-66.3, 26.8	-16.7, 29.0
Week 52		
n	31	26
Mean (SD)	62.70 (19.64)	63.23 (17.08)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	66.67	62.16
25th, 75th Percentile	51.39, 80.90	48.96, 79.17
Min, Max	9.0, 91.3	26.0, 91.3
Change from baseline to Week 52 ^a		
n	22	16
Mean (SD)	-3.86 (20.89)	6.67 (16.35)
Median	-3.30	2.26
25th, 75th Percentile	-13.54, 9.38	-2.43, 13.20
Min, Max	-63.9, 28.5	-15.8, 41.0
Difference in change from baseline (95%CI)		10.53 (-2.22, 23.27)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.1027
Hedges'g (95% CI) ^c		0.54 (-0.12, 1.19)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported QoLISSY : Total Score		
Baseline		
n	13	12
Mean (SD)	65.25 (20.51)	73.18 (14.88)
Median	71.88	76.74
25th, 75th Percentile	51.74, 80.21	65.98, 82.30
Min, Max	21.2, 90.6	37.5, 92.0
Week 26		
n	13	11
Mean (SD)	70.33 (14.22)	74.90 (9.64)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.27	74.65
25th, 75th Percentile	64.58, 78.13	67.71, 83.33
Min, Max	34.4, 87.9	59.7, 90.3
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	5.08 (11.75)	0.94 (16.17)
Median	6.59	0.34
25th, 75th Percentile	-4.86, 13.20	-6.95, 10.42
Min, Max	-11.1, 29.9	-32.3, 25.0
Week 52		
n	13	10
Mean (SD)	69.39 (17.74)	78.22 (7.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.35	80.39
25th, 75th Percentile	59.03, 80.21	73.12, 81.94
Min, Max	31.9, 91.0	61.8, 87.9
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	4.15 (14.75)	0.61 (10.34)
Median	2.78	-1.82
25th, 75th Percentile	-1.04, 9.03	-5.55, 5.91
Min, Max	-25.6, 39.2	-11.1, 24.3
Difference in change from baseline (95%CI)		-3.54 (-14.95, 7.88)
P-value ^b		0.5264

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.26 (-1.09, 0.57)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.1299

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported QoLISSY : Total Score		
Baseline		
n	4	9
Mean (SD)	61.22 (11.07)	63.47 (10.69)
Median	61.46	60.42
25th, 75th Percentile	52.26, 70.18	57.29, 71.18
Min, Max	48.6, 73.3	49.3, 80.6
Week 26		
n	6	8
Mean (SD)	53.53 (12.43)	70.08 (16.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	51.33	69.62
25th, 75th Percentile	44.20, 56.60	66.15, 81.95
Min, Max	41.7, 76.0	35.8, 89.4
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-5.50 (17.33)	14.45 (13.34)
Median	-0.93	12.85
25th, 75th Percentile	-18.39, 7.40	9.38, 28.82
Min, Max	-29.1, 9.0	-6.3, 29.0
Week 52		
n	6	10
Mean (SD)	54.98 (9.37)	77.04 (11.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	54.69	82.27
25th, 75th Percentile	50.70, 59.72	69.45, 84.73
Min, Max	41.3, 68.8	51.0, 91.3
Change from baseline to Week 52 ^a		
n	4	7
Mean (SD)	-1.93 (9.81)	14.97 (14.61)
Median	-1.74	10.07
25th, 75th Percentile	-9.41, 5.56	-1.04, 30.90
Min, Max	-13.6, 9.4	-1.4, 33.6
Difference in change from baseline (95%CI)		16.90 (-1.82, 35.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.0716
Hedges'g (95% CI) ^c		1.17 (-0.20, 2.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported QoLISSY : Total Score		
Baseline		
n	14	9
Mean (SD)	70.86 (15.43)	63.05 (18.83)
Median	73.79	66.32
25th, 75th Percentile	59.03, 83.68	47.22, 73.61
Min, Max	41.7, 90.6	32.3, 88.9
Week 26		
n	13	9
Mean (SD)	72.16 (11.91)	63.33 (19.94)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.23	69.05
25th, 75th Percentile	64.58, 80.56	44.79, 76.24
Min, Max	55.6, 92.7	33.3, 95.1
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	0.40 (11.92)	3.00 (12.27)
Median	-3.47	3.05
25th, 75th Percentile	-6.60, 1.74	-2.96, 7.30
Min, Max	-11.1, 29.9	-16.7, 25.9
Week 52		
n	17	12
Mean (SD)	67.83 (20.51)	58.57 (20.15)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	76.74	49.14
25th, 75th Percentile	59.03, 80.90	47.57, 80.04
Min, Max	9.0, 88.9	26.0, 90.6
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	1.64 (15.16)	2.39 (7.94)
Median	4.86	1.74
25th, 75th Percentile	-3.48, 9.03	-0.69, 6.18
Min, Max	-35.8, 25.7	-12.2, 16.3
Difference in change from baseline (95%CI)		0.75 (-10.71, 12.21)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8929
Hedges'g (95% CI) ^c		0.06 (-0.78, 0.89)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported QoLISSY : Total Score		
Baseline		
n	12	8
Mean (SD)	62.58 (17.77)	59.92 (23.52)
Median	64.76	65.45
25th, 75th Percentile	52.78, 70.27	41.84, 76.12
Min, Max	21.2, 90.3	20.5, 92.0
Week 26		
n	14	11
Mean (SD)	64.95 (19.33)	66.67 (14.52)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.78	71.53
25th, 75th Percentile	55.56, 78.82	59.73, 74.01
Min, Max	24.0, 93.1	41.7, 90.3
Change from baseline to Week 26 ^a		
n	12	8
Mean (SD)	-0.05 (21.99)	3.72 (19.76)
Median	6.42	3.13
25th, 75th Percentile	-1.07, 11.51	-6.65, 22.05
Min, Max	-66.3, 14.2	-32.3, 25.0
Week 52		
n	16	10
Mean (SD)	63.76 (20.66)	66.79 (11.84)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	66.84	64.42
25th, 75th Percentile	50.70, 81.78	59.38, 74.31
Min, Max	26.4, 91.3	49.0, 87.9
Change from baseline to Week 52 ^a		
n	12	6
Mean (SD)	-3.96 (27.46)	3.56 (18.70)
Median	-1.56	-4.07
25th, 75th Percentile	-19.57, 12.15	-5.55, 2.78
Min, Max	-63.9, 39.2	-8.7, 41.0
Difference in change from baseline (95%CI)		7.52 (-19.03, 34.07)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.5
 Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.5567
Hedges'g (95% CI) ^c		0.29 (-0.70, 1.27)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported QoLISSY : Total Score		
Baseline		
n	6	4
Mean (SD)	67.08 (17.44)	79.95 (7.83)
Median	71.03	81.60
25th, 75th Percentile	61.11, 80.21	74.66, 85.25
Min, Max	36.1, 83.0	69.1, 87.5
Week 26		
n	6	4
Mean (SD)	73.50 (12.26)	74.57 (8.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.44	74.83
25th, 75th Percentile	69.79, 78.13	67.19, 81.95
Min, Max	54.2, 92.0	65.3, 83.3
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	6.42 (13.84)	-5.38 (4.84)
Median	2.71	-5.38
25th, 75th Percentile	-4.86, 18.06	-9.03, -1.74
Min, Max	-6.9, 26.8	-11.1, 0.3
Week 52		
n	5	4
Mean (SD)	68.54 (18.48)	71.24 (12.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.001.005_qs_sum_ovr_qol_self_tot_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	75.35	75.18
25th, 75th Percentile	66.67, 79.17	62.61, 79.86
Min, Max	37.5, 84.0	53.3, 81.3
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	0.27 (11.41)	-8.72 (7.09)
Median	-1.04	-10.07
25th, 75th Percentile	-7.64, 1.39	-13.44, -4.00
Min, Max	-10.2, 18.9	-15.8, 1.0
Difference in change from baseline (95%CI)		-8.99 (-24.52, 6.55)
P-value ^b		0.2137

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.2.1.5
 Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Total Score for BMN111-301
 Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		-0.82 (-2.17, 0.59)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.3937

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported QoLISSY : Total Score		
Baseline		
n	13	10
Mean (SD)	67.27 (19.41)	67.55 (12.71)
Median	71.88	67.71
25th, 75th Percentile	55.90, 76.88	59.38, 75.15
Min, Max	21.2, 90.6	47.2, 88.9
Week 26		
n	14	10
Mean (SD)	62.17 (17.62)	68.41 (15.56)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	64.93	69.45
25th, 75th Percentile	54.38, 76.04	64.93, 76.74
Min, Max	24.0, 83.3	35.8, 95.1
Change from baseline to Week 26 ^a		
n	13	7
Mean (SD)	-4.67 (20.36)	4.05 (8.56)
Median	-3.27	6.25
25th, 75th Percentile	-7.63, 6.59	-3.82, 9.03
Min, Max	-66.3, 13.2	-10.2, 15.3
Week 52		
n	15	10
Mean (SD)	59.56 (23.45)	67.24 (15.66)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	67.71	66.15
25th, 75th Percentile	41.32, 78.13	53.34, 80.56
Min, Max	9.0, 91.0	46.5, 90.6
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-2.42 (23.01)	-1.32 (8.29)
Median	1.73	-0.87
25th, 75th Percentile	-5.20, 6.25	-6.08, 4.52
Min, Max	-63.9, 39.2	-15.8, 10.1
Difference in change from baseline (95%CI)		1.10 (-13.76, 15.96)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.8777
Hedges'g (95% CI) ^c		0.06 (-0.83, 0.94)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported QoLISSY : Total Score		
Baseline		
n	14	9
Mean (SD)	67.19 (14.78)	56.68 (22.88)
Median	67.02	60.42
25th, 75th Percentile	58.34, 80.21	43.13, 71.18
Min, Max	41.7, 86.1	20.5, 92.0
Week 26		
n	12	12
Mean (SD)	70.92 (9.07)	61.65 (17.03)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	72.23	66.99
25th, 75th Percentile	64.59, 76.17	44.62, 72.57
Min, Max	55.9, 87.9	33.3, 89.4
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	5.58 (10.94)	4.41 (22.65)
Median	4.87	1.04
25th, 75th Percentile	-4.86, 14.23	-11.11, 25.92
Min, Max	-6.9, 29.9	-32.3, 29.0
Week 52		
n	14	12
Mean (SD)	69.40 (15.60)	65.59 (17.33)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	77.26	64.24
25th, 75th Percentile	52.43, 81.60	49.14, 82.10
Min, Max	41.0, 88.9	39.2, 91.3
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	3.04 (17.00)	12.37 (18.91)
Median	2.78	6.18
25th, 75th Percentile	-3.82, 12.84	-1.39, 30.90
Min, Max	-35.8, 28.5	-12.2, 41.0
Difference in change from baseline (95%CI)		9.33 (-6.76, 25.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.2407
Hedges'g (95% CI) ^c		0.50 (-0.37, 1.36)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported QoLISSY : Total Score		
Baseline		
n	9	11
Mean (SD)	63.92 (14.07)	68.37 (15.83)
Median	65.18	73.61
25th, 75th Percentile	59.03, 73.34	57.29, 81.60
Min, Max	36.1, 85.1	37.5, 87.5
Week 26		
n	13	10
Mean (SD)	68.33 (19.06)	73.85 (13.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	60.71	75.45
25th, 75th Percentile	55.21, 84.38	67.71, 83.33
Min, Max	41.7, 93.1	43.4, 90.3
Change from baseline to Week 26 ^a		
n	9	10
Mean (SD)	2.18 (16.30)	5.09 (11.59)
Median	2.78	1.49
25th, 75th Percentile	-4.92, 9.82	-3.08, 10.42
Min, Max	-29.1, 26.8	-7.0, 25.0
Week 52		
n	15	14
Mean (SD)	65.39 (17.29)	69.04 (17.18)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.97	73.72
25th, 75th Percentile	51.39, 81.95	61.81, 81.60
Min, Max	34.7, 91.3	26.0, 86.1
Change from baseline to Week 52 ^a		
n	9	9
Mean (SD)	-4.35 (16.37)	1.34 (10.83)
Median	-3.12	0.34
25th, 75th Percentile	-13.62, 5.21	-5.55, 5.91
Min, Max	-29.2, 18.9	-11.1, 24.3
Difference in change from baseline (95%CI)		5.68 (-8.19, 19.55)
P-value ^b		0.3979

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		0.39 (-0.55, 1.32)
P-value for interaction term, treatment * [Baseline AGV]		0.7461

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported QoLISSY : Total Score		
Baseline		
n	25	24
Mean (SD)	65.10 (17.28)	65.35 (16.93)
Median	65.63	67.71
25th, 75th Percentile	53.13, 76.74	50.70, 78.83
Min, Max	21.2, 90.6	32.3, 92.0
Week 26		
n	27	27
Mean (SD)	66.28 (16.79)	66.15 (14.92)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	69.79	69.05
25th, 75th Percentile	55.56, 78.82	59.73, 76.24
Min, Max	24.0, 92.7	33.3, 95.1
Change from baseline to Week 26 ^a		
n	23	21
Mean (SD)	-0.19 (18.38)	2.26 (14.43)
Median	2.78	1.04
25th, 75th Percentile	-5.21, 9.02	-6.25, 9.03
Min, Max	-66.3, 26.8	-32.3, 28.8
Week 52		
n	30	27
Mean (SD)	64.42 (21.72)	67.23 (15.03)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	68.23	69.79
25th, 75th Percentile	52.43, 83.33	51.04, 80.56
Min, Max	9.0, 91.3	39.2, 90.6
Change from baseline to Week 52 ^a		
n	24	21
Mean (SD)	-0.41 (22.04)	1.77 (12.01)
Median	2.08	-0.69
25th, 75th Percentile	-10.07, 12.15	-4.17, 6.18
Min, Max	-63.9, 39.2	-15.8, 33.6
Difference in change from baseline (95%CI)		2.17 (-8.38, 12.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
P-value ^b		0.6790
Hedges'g (95% CI) ^c		0.12 (-0.47, 0.70)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported QoLISSY : Total Score		
Baseline		
n	11	6
Mean (SD)	69.36 (13.11)	61.58 (21.44)
Median	74.56	65.98
25th, 75th Percentile	59.03, 80.21	60.42, 76.39
Min, Max	43.1, 85.1	20.5, 80.2
Week 26		
n	12	5
Mean (SD)	68.35 (14.98)	75.25 (20.64)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	73.10	85.77
25th, 75th Percentile	57.30, 76.92	69.10, 89.44
Min, Max	41.7, 93.1	41.7, 90.3
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	2.46 (12.78)	14.28 (15.88)
Median	-3.37	21.18
25th, 75th Percentile	-6.94, 9.82	9.38, 22.92
Min, Max	-11.1, 29.9	-11.1, 29.0
Week 52		
n	14	9
Mean (SD)	65.23 (12.63)	67.86 (21.06)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Median	65.46	69.45
25th, 75th Percentile	52.09, 79.17	61.46, 84.73
Min, Max	49.0, 82.0	26.0, 91.3
Change from baseline to Week 52 ^a		
n	11	5
Mean (SD)	-1.93 (10.53)	15.14 (19.93)
Median	-1.04	8.34
25th, 75th Percentile	-7.64, 5.21	1.04, 30.90
Min, Max	-25.6, 12.8	-5.6, 41.0
Difference in change from baseline (95%CI)		17.07 (1.01, 33.13)
P-value ^b		0.0388

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.1.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Hedges'g (95% CI) ^c		1.16 (0.00, 2.28)
P-value for interaction term, treatment * [Ethnicity]		0.1643

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	20	16
Mean (SD)	63.33 (18.81)	52.29 (22.38)
Median	66.67	54.17
25th, 75th Percentile	47.92, 77.09	33.34, 70.84
Min, Max	33.3, 95.8	16.7, 91.7
Week 26		
n	20	16
Mean (SD)	55.21 (15.70)	56.77 (21.13)
Median	56.25	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	47.92, 66.67	39.59, 70.84
Min, Max	29.2, 79.2	25.0, 95.8
Change from baseline to Week 26 ^a		
n	18	15
Mean (SD)	-7.17 (20.70)	6.72 (17.60)
Median	-8.33	4.17
25th, 75th Percentile	-16.67, 8.33	-8.33, 16.67
Min, Max	-45.8, 41.7	-20.8, 37.5
Week 52		
n	25	19
Mean (SD)	60.53 (19.80)	56.01 (15.69)
Median	62.50	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.2.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 75.00	45.83, 66.67
Min, Max	8.3, 83.3	20.8, 87.5
Change from baseline to Week 52 ^a		
n	20	15
Mean (SD)	-2.29 (24.39)	6.00 (15.81)
Median	-2.09	4.16
25th, 75th Percentile	-14.59, 12.50	-4.17, 20.83
Min, Max	-66.7, 45.8	-19.2, 41.7
Difference in change from baseline (95%CI)		8.29 (-6.43, 23.01)
P-value ^b		0.2600
Hedges'g (95% CI) ^c		0.38 (-0.30, 1.06)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	17	14
Mean (SD)	58.14 (15.93)	61.01 (17.19)
Median	60.00	58.34
25th, 75th Percentile	50.00, 70.00	50.00, 75.00
Min, Max	29.2, 83.3	37.5, 91.7
Week 26		
n	20	17
Mean (SD)	60.92 (21.71)	64.66 (19.77)
Median	68.75	70.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 75.00	54.17, 75.00
Min, Max	25.0, 91.7	16.7, 100.0
Change from baseline to Week 26 ^a		
n	17	12
Mean (SD)	3.97 (18.54)	6.53 (27.17)
Median	4.17	6.26
25th, 75th Percentile	-4.17, 12.50	-8.34, 22.92
Min, Max	-45.0, 33.3	-50.0, 62.5
Week 52		
n	20	18
Mean (SD)	61.04 (18.35)	67.41 (17.32)
Median	62.50	72.92

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 70.83	54.17, 79.17
Min, Max	29.2, 91.7	25.0, 91.7
Change from baseline to Week 52 ^a		
n	17	12
Mean (SD)	2.40 (16.81)	7.64 (20.09)
Median	4.16	2.08
25th, 75th Percentile	0.00, 10.83	-6.25, 16.67
Min, Max	-41.7, 33.3	-16.7, 45.8
Difference in change from baseline (95%CI)		5.24 (-8.86, 19.33)
P-value ^b		0.4524
Hedges'g (95% CI) ^c		0.28 (-0.47, 1.02)
P-value for interaction term, treatment * [Sex]		0.7639

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	0	2
Mean (SD)		43.75 (26.52)
Median		43.75
25th, 75th Percentile		25.00, 62.50
Min, Max		25.0, 62.5
Week 26		
n	5	7
Mean (SD)	51.67 (22.55)	48.81 (23.78)
Median	66.67	45.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.17, 66.67	25.00, 70.83
Min, Max	25.0, 70.8	16.7, 75.0
Change from baseline to Week 26 ^a		
n	0	1
Mean (SD)		0.00 (NA)
Median		0.00
25th, 75th Percentile		0.00, 0.00
Min, Max		0.0, 0.0
Week 52		
n	8	11
Mean (SD)	60.52 (24.59)	57.27 (17.89)
Median	68.75	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 77.50	45.83, 70.83
Min, Max	8.3, 83.3	25.0, 80.0
Change from baseline to Week 52 ^a		
n	0	2
Mean (SD)		14.58 (8.84)
Median		14.58
25th, 75th Percentile		8.33, 20.83
Min, Max		8.3, 20.8
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	24	16
Mean (SD)	59.58 (18.95)	53.59 (19.85)
Median	61.25	52.09
25th, 75th Percentile	43.75, 72.50	39.59, 70.84
Min, Max	29.2, 95.8	20.8, 91.7
Week 26		
n	23	15
Mean (SD)	57.32 (20.37)	64.39 (18.15)
Median	54.17	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 75.00	45.83, 79.17
Min, Max	25.0, 91.7	37.5, 100.0
Change from baseline to Week 26 ^a		
n	23	15
Mean (SD)	-1.96 (22.86)	10.55 (21.38)
Median	-4.16	4.17
25th, 75th Percentile	-12.50, 8.34	-8.34, 25.00
Min, Max	-45.8, 41.7	-12.5, 62.5
Week 52		
n	24	16
Mean (SD)	58.51 (17.00)	63.54 (16.21)
Median	58.33	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 68.75	50.00, 79.17
Min, Max	29.2, 91.7	37.5, 87.5
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	-1.08 (23.39)	10.06 (18.03)
Median	2.08	4.17
25th, 75th Percentile	-16.25, 11.67	-3.33, 25.00
Min, Max	-66.7, 45.8	-16.7, 45.8
Difference in change from baseline (95%CI)		11.13 (-3.22, 25.48)
P-value ^b		0.1245
Hedges'g (95% CI) ^c		0.51 (-0.15, 1.16)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	13	12
Mean (SD)	63.46 (14.84)	62.15 (20.14)
Median	62.50	62.50
25th, 75th Percentile	54.17, 75.00	50.00, 77.09
Min, Max	37.5, 87.5	16.7, 91.7
Week 26		
n	12	11
Mean (SD)	62.15 (14.70)	63.64 (20.42)
Median	66.67	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	52.08, 72.92	41.67, 79.17
Min, Max	37.5, 79.2	29.2, 95.8
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	-1.39 (14.69)	1.89 (23.52)
Median	0.00	8.33
25th, 75th Percentile	-10.42, 8.34	-8.33, 12.50
Min, Max	-33.3, 20.8	-50.0, 37.5
Week 52		
n	13	10
Mean (SD)	65.06 (19.51)	63.09 (19.15)
Median	70.83	66.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 79.17	54.17, 75.00
Min, Max	29.2, 87.5	20.8, 91.7
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	1.60 (16.80)	0.17 (17.02)
Median	0.00	-4.16
25th, 75th Percentile	-4.17, 12.50	-8.33, 4.16
Min, Max	-41.7, 25.0	-19.2, 41.7
Difference in change from baseline (95%CI)		-1.43 (-16.21, 13.34)
P-value ^b		0.8420
Hedges'g (95% CI) ^c		-0.08 (-0.91, 0.74)
P-value for interaction term, treatment * [Age at Baseline]		0.2402

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	24	18
Mean (SD)	61.84 (17.55)	48.80 (20.31)
Median	62.50	47.92
25th, 75th Percentile	47.92, 72.92	37.50, 62.50
Min, Max	33.3, 95.8	16.7, 91.7
Week 26		
n	27	22
Mean (SD)	55.62 (19.02)	54.70 (20.64)
Median	58.33	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 70.83	41.67, 70.83
Min, Max	25.0, 91.7	16.7, 100.0
Change from baseline to Week 26 ^a		
n	22	16
Mean (SD)	-5.07 (21.45)	8.59 (21.35)
Median	-4.17	4.17
25th, 75th Percentile	-12.50, 8.34	-8.34, 18.75
Min, Max	-45.8, 41.7	-20.8, 62.5
Week 52		
n	32	27
Mean (SD)	59.27 (19.04)	56.57 (16.73)
Median	62.50	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	47.92, 70.83	45.83, 66.67
Min, Max	8.3, 87.5	20.8, 87.5
Change from baseline to Week 52 ^a		
n	24	17
Mean (SD)	-2.99 (24.15)	8.48 (18.08)
Median	0.00	8.33
25th, 75th Percentile	-16.25, 11.67	-3.33, 20.83
Min, Max	-66.7, 45.8	-19.2, 45.8
Difference in change from baseline (95%CI)		11.47 (-2.56, 25.49)
P-value ^b		0.1062
Hedges'g (95% CI) ^c		0.51 (-0.12, 1.14)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	13	12
Mean (SD)	59.29 (18.01)	67.71 (14.56)
Median	58.33	72.92
25th, 75th Percentile	50.00, 75.00	52.09, 77.09
Min, Max	29.2, 87.5	50.0, 91.7
Week 26		
n	13	11
Mean (SD)	63.14 (18.39)	73.11 (14.23)
Median	66.67	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 75.00	66.67, 79.17
Min, Max	25.0, 87.5	41.7, 95.8
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	3.85 (17.22)	3.79 (23.38)
Median	4.17	4.17
25th, 75th Percentile	-8.33, 8.34	-8.33, 25.00
Min, Max	-33.3, 33.3	-50.0, 37.5
Week 52		
n	13	10
Mean (SD)	64.42 (18.99)	75.00 (10.39)
Median	62.50	77.09

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.2.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 79.17	66.67, 79.17
Min, Max	33.3, 91.7	54.2, 91.7
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	5.13 (13.08)	3.75 (16.95)
Median	4.16	-2.08
25th, 75th Percentile	-4.17, 12.50	-8.33, 4.17
Min, Max	-16.7, 33.3	-12.5, 41.7
Difference in change from baseline (95%CI)		-1.38 (-14.38, 11.62)
P-value ^b		0.8279
Hedges'g (95% CI) ^c		-0.09 (-0.91, 0.74)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.2207

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	4	9
Mean (SD)	47.71 (17.82)	53.24 (10.98)
Median	43.75	54.17
25th, 75th Percentile	33.33, 62.09	50.00, 62.50
Min, Max	33.3, 70.0	37.5, 66.7
Week 26		
n	6	8
Mean (SD)	34.31 (14.29)	63.02 (23.72)
Median	29.17	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 35.00	56.25, 75.00
Min, Max	25.0, 62.5	16.7, 100.0
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-9.79 (24.02)	19.44 (24.81)
Median	-1.25	16.67
25th, 75th Percentile	-24.58, 5.00	8.33, 25.00
Min, Max	-45.0, 8.3	-12.5, 62.5
Week 52		
n	6	10
Mean (SD)	49.31 (10.01)	68.83 (16.41)
Median	50.00	70.83

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.83, 54.17	58.33, 80.00
Min, Max	33.3, 62.5	37.5, 91.7
Change from baseline to Week 52 ^a		
n	4	7
Mean (SD)	1.25 (12.52)	16.67 (22.44)
Median	4.17	12.50
25th, 75th Percentile	-7.92, 10.42	0.00, 41.67
Min, Max	-15.8, 12.5	-16.7, 45.8
Difference in change from baseline (95%CI)		15.42 (-12.51, 43.34)
P-value ^b		0.2432
Hedges'g (95% CI) ^c		0.72 (-0.57, 1.97)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	15	9
Mean (SD)	65.11 (14.79)	52.69 (24.68)
Median	66.67	45.83
25th, 75th Percentile	58.33, 75.00	41.67, 70.83
Min, Max	37.5, 87.5	16.7, 91.7
Week 26		
n	14	10
Mean (SD)	66.37 (14.01)	55.75 (22.71)
Median	68.75	52.08

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.33, 75.00	37.50, 75.00
Min, Max	37.5, 87.5	25.0, 91.7
Change from baseline to Week 26 ^a		
n	13	9
Mean (SD)	0.83 (18.44)	5.09 (13.21)
Median	4.17	0.00
25th, 75th Percentile	-12.50, 8.34	-0.83, 12.50
Min, Max	-33.3, 33.3	-12.5, 30.0
Week 52		
n	17	12
Mean (SD)	61.76 (21.41)	51.74 (21.13)
Median	66.67	47.92

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.33, 70.83	37.50, 66.67
Min, Max	8.3, 87.5	20.8, 87.5
Change from baseline to Week 52 ^a		
n	15	9
Mean (SD)	-0.11 (17.46)	4.72 (11.61)
Median	4.16	4.16
25th, 75th Percentile	-8.34, 12.50	-4.16, 8.34
Min, Max	-41.7, 25.0	-8.3, 25.0
Difference in change from baseline (95%CI)		4.83 (-8.79, 18.46)
P-value ^b		0.4697
Hedges'g (95% CI) ^c		0.30 (-0.53, 1.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	12	8
Mean (SD)	59.72 (17.88)	53.13 (23.44)
Median	60.42	52.09
25th, 75th Percentile	50.00, 66.67	37.50, 66.67
Min, Max	29.2, 95.8	20.8, 91.7
Week 26		
n	14	11
Mean (SD)	56.85 (18.61)	60.61 (19.49)
Median	56.25	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 70.83	41.67, 75.00
Min, Max	25.0, 91.7	37.5, 95.8
Change from baseline to Week 26 ^a		
n	12	8
Mean (SD)	-4.51 (20.75)	6.25 (28.70)
Median	-4.17	10.42
25th, 75th Percentile	-10.42, 8.34	-8.34, 29.17
Min, Max	-45.8, 25.0	-50.0, 37.5
Week 52		
n	16	11
Mean (SD)	61.77 (20.47)	62.88 (11.41)
Median	64.59	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.84, 79.59	54.17, 75.00
Min, Max	29.2, 91.7	45.8, 79.2
Change from baseline to Week 52 ^a		
n	12	7
Mean (SD)	-3.12 (27.07)	8.33 (18.00)
Median	0.00	4.17
25th, 75th Percentile	-8.34, 8.34	-4.16, 20.83
Min, Max	-66.7, 33.3	-12.5, 41.7
Difference in change from baseline (95%CI)		11.46 (-12.88, 35.80)
P-value ^b		0.3346
Hedges'g (95% CI) ^c		0.45 (-0.50, 1.39)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	6	4
Mean (SD)	61.80 (22.42)	78.13 (3.99)
Median	66.67	77.09
25th, 75th Percentile	37.50, 83.33	75.00, 81.25
Min, Max	33.3, 83.3	75.0, 83.3
Week 26		
n	6	4
Mean (SD)	65.28 (15.52)	69.79 (12.44)
Median	62.50	66.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 79.17	62.50, 77.09
Min, Max	50.0, 87.5	58.3, 87.5
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	3.48 (23.34)	-8.33 (10.21)
Median	-2.08	-8.33
25th, 75th Percentile	-8.33, 16.67	-14.59, -2.08
Min, Max	-25.0, 41.7	-20.8, 4.2
Week 52		
n	6	4
Mean (SD)	66.67 (11.49)	69.17 (7.26)
Median	64.59	70.84

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 75.00	63.34, 75.00
Min, Max	50.0, 83.3	60.0, 75.0
Change from baseline to Week 52 ^a		
n	6	4
Mean (SD)	4.86 (24.35)	-8.96 (7.86)
Median	2.09	-8.33
25th, 75th Percentile	-16.66, 16.67	-13.75, -4.17
Min, Max	-20.8, 45.8	-19.2, 0.0
Difference in change from baseline (95%CI)		-13.82 (-43.36, 15.72)
P-value ^b		0.3121
Hedges'g (95% CI) ^c		-0.63 (-1.91, 0.69)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.3535

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	13	10
Mean (SD)	64.74 (22.02)	51.67 (23.51)
Median	70.83	47.92
25th, 75th Percentile	54.17, 83.33	37.50, 66.67
Min, Max	29.2, 95.8	16.7, 91.7
Week 26		
n	14	11
Mean (SD)	52.80 (19.26)	54.85 (21.10)
Median	54.17	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 70.83	41.67, 70.83
Min, Max	25.0, 79.2	16.7, 91.7
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-10.13 (16.29)	6.67 (17.43)
Median	-8.33	6.25
25th, 75th Percentile	-16.67, 1.67	-4.58, 18.75
Min, Max	-45.8, 8.3	-20.8, 33.3
Week 52		
n	15	11
Mean (SD)	55.55 (23.18)	58.94 (19.76)
Median	62.50	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	33.33, 66.67	45.83, 75.00
Min, Max	8.3, 91.7	20.8, 87.5
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	-4.81 (23.99)	4.81 (13.19)
Median	0.00	4.16
25th, 75th Percentile	-16.67, 8.33	0.00, 8.34
Min, Max	-66.7, 33.3	-19.2, 25.0
Difference in change from baseline (95%CI)		9.62 (-8.80, 28.04)
P-value ^b		0.2890
Hedges'g (95% CI) ^c		0.45 (-0.41, 1.31)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	14	9
Mean (SD)	61.61 (13.49)	52.69 (23.23)
Median	62.50	54.17
25th, 75th Percentile	50.00, 70.83	37.50, 66.67
Min, Max	37.5, 83.3	20.8, 91.7
Week 26		
n	13	12
Mean (SD)	65.06 (11.73)	55.21 (20.73)
Median	66.67	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.33, 70.83	39.59, 68.75
Min, Max	45.8, 87.5	25.0, 100.0
Change from baseline to Week 26 ^a		
n	12	9
Mean (SD)	3.47 (14.08)	3.80 (31.44)
Median	2.09	0.00
25th, 75th Percentile	-8.33, 10.42	-12.50, 16.67
Min, Max	-12.5, 33.3	-50.0, 62.5
Week 52		
n	15	12
Mean (SD)	63.89 (16.34)	58.68 (15.54)
Median	66.67	58.34

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.17, 75.00	47.92, 70.84
Min, Max	29.2, 87.5	33.3, 83.3
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	2.08 (18.47)	8.89 (23.00)
Median	4.17	0.00
25th, 75th Percentile	-8.34, 12.50	-8.33, 20.83
Min, Max	-41.7, 29.2	-16.7, 45.8
Difference in change from baseline (95%CI)		6.81 (-11.24, 24.85)
P-value ^b		0.4417
Hedges'g (95% CI) ^c		0.32 (-0.52, 1.16)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	10	11
Mean (SD)	55.08 (16.01)	63.64 (13.06)
Median	55.00	58.33
25th, 75th Percentile	41.67, 66.67	50.00, 75.00
Min, Max	33.3, 83.3	50.0, 83.3
Week 26		
n	13	10
Mean (SD)	56.73 (23.17)	74.17 (13.86)
Median	54.17	77.09

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 75.00	66.67, 79.17
Min, Max	25.0, 91.7	45.8, 95.8
Change from baseline to Week 26 ^a		
n	10	10
Mean (SD)	2.84 (28.01)	9.17 (15.93)
Median	8.34	6.26
25th, 75th Percentile	-12.50, 25.00	-8.33, 25.00
Min, Max	-45.0, 41.7	-8.3, 37.5
Week 52		
n	15	14
Mean (SD)	62.83 (16.76)	66.07 (16.89)
Median	58.33	66.67

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.2.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 80.00	58.33, 79.17
Min, Max	29.2, 83.3	25.0, 91.7
Change from baseline to Week 52 ^a		
n	10	9
Mean (SD)	2.83 (21.72)	6.48 (16.81)
Median	0.00	4.16
25th, 75th Percentile	-4.17, 12.50	-4.16, 8.33
Min, Max	-37.5, 45.8	-8.3, 41.7
Difference in change from baseline (95%CI)		3.65 (-15.31, 22.61)
P-value ^b		0.6897
Hedges'g (95% CI) ^c		0.18 (-0.73, 1.08)
P-value for interaction term, treatment * [Baseline AGV]		0.8957

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.2.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	26	24
Mean (SD)	60.45 (17.43)	58.13 (20.67)
Median	62.50	56.25
25th, 75th Percentile	50.00, 70.83	45.42, 75.00
Min, Max	29.2, 95.8	16.7, 91.7
Week 26		
n	28	28
Mean (SD)	56.46 (17.80)	58.15 (18.84)
Median	58.33	58.33

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.2.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	47.92, 68.75	45.42, 72.92
Min, Max	25.0, 87.5	16.7, 91.7
Change from baseline to Week 26 ^a		
n	25	22
Mean (SD)	-3.63 (19.90)	2.08 (18.60)
Median	-4.17	2.09
25th, 75th Percentile	-12.50, 8.33	-8.34, 12.50
Min, Max	-45.8, 41.7	-50.0, 33.3
Week 52		
n	31	28
Mean (SD)	59.70 (21.74)	61.55 (17.25)
Median	62.50	64.59

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.2.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	41.67, 79.17	50.00, 75.00
Min, Max	8.3, 91.7	20.8, 91.7
Change from baseline to Week 52 ^a		
n	26	22
Mean (SD)	-0.35 (24.24)	3.33 (14.57)
Median	2.08	2.08
25th, 75th Percentile	-12.50, 12.50	-8.33, 8.34
Min, Max	-66.7, 45.8	-19.2, 41.7
Difference in change from baseline (95%CI)		3.69 (-7.78, 15.15)
P-value ^b		0.5198
Hedges'g (95% CI) ^c		0.18 (-0.39, 0.75)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.2.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported QoLISSY : Physical Score		
Baseline		
n	11	6
Mean (SD)	62.12 (18.49)	49.31 (18.53)
Median	66.67	52.09
25th, 75th Percentile	45.83, 83.33	37.50, 58.33
Min, Max	33.3, 83.3	20.8, 75.0
Week 26		
n	12	5
Mean (SD)	61.81 (21.67)	75.83 (25.24)
Median	68.75	79.17

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	45.84, 75.00	66.67, 95.83
Min, Max	25.0, 91.7	37.5, 100.0
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	2.92 (21.25)	26.67 (26.12)
Median	6.26	25.00
25th, 75th Percentile	-8.33, 20.83	16.67, 37.50
Min, Max	-33.3, 33.3	-8.3, 62.5
Week 52		
n	14	9
Mean (SD)	63.10 (10.82)	61.57 (18.37)
Median	62.50	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.2.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Physical Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.17, 70.83	54.17, 75.00
Min, Max	45.8, 83.3	25.0, 83.3
Change from baseline to Week 52 ^a		
n	11	5
Mean (SD)	0.38 (11.55)	21.67 (23.08)
Median	0.00	25.00
25th, 75th Percentile	-8.33, 12.50	0.00, 41.67
Min, Max	-20.8, 12.5	-4.2, 45.8
Difference in change from baseline (95%CI)		21.29 (3.09, 39.49)
P-value ^b		0.0251
Hedges'g (95% CI) ^c		1.28 (0.10, 2.42)
P-value for interaction term, treatment * [Ethnicity]		0.1438

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported QoLISSY : Social Score		
Baseline		
n	20	16
Mean (SD)	68.71 (21.51)	64.48 (21.85)
Median	71.88	67.19
25th, 75th Percentile	50.00, 85.94	50.01, 81.48
Min, Max	34.4, 100.0	18.8, 96.9
Week 26		
n	20	16
Mean (SD)	69.96 (21.87)	64.84 (20.71)
Median	75.00	70.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.3.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.69, 85.94	48.44, 75.00
Min, Max	12.5, 96.9	31.3, 100.0
Change from baseline to Week 26 ^a		
n	18	15
Mean (SD)	0.52 (25.43)	3.93 (15.13)
Median	-3.13	3.12
25th, 75th Percentile	-6.25, 12.50	-6.69, 12.50
Min, Max	-84.4, 32.6	-15.6, 34.4
Week 52		
n	25	18
Mean (SD)	62.25 (28.77)	62.55 (16.94)
Median	65.63	60.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 84.38	50.00, 78.13
Min, Max	0.0, 100.0	37.5, 90.6
Change from baseline to Week 52 ^a		
n	20	14
Mean (SD)	-4.33 (28.13)	1.85 (18.23)
Median	-4.69	-3.12
25th, 75th Percentile	-14.29, 14.07	-6.25, 6.25
Min, Max	-81.3, 37.5	-28.1, 41.5
Difference in change from baseline (95%CI)		6.18 (-11.28, 23.64)
P-value ^b		0.4762
Hedges'g (95% CI) ^c		0.25 (-0.44, 0.93)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported QoLISSY : Social Score		
Baseline		
n	17	14
Mean (SD)	67.20 (19.89)	67.86 (18.11)
Median	65.63	68.75
25th, 75th Percentile	59.38, 84.38	62.50, 75.00
Min, Max	28.1, 96.9	21.9, 96.9
Week 26		
n	20	16
Mean (SD)	71.85 (17.42)	67.55 (17.10)
Median	71.88	68.53

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.3.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.26, 82.82	53.13, 79.69
Min, Max	43.8, 100.0	40.6, 96.9
Change from baseline to Week 26 ^a		
n	17	11
Mean (SD)	6.67 (15.13)	-0.89 (21.00)
Median	3.12	3.13
25th, 75th Percentile	-3.13, 18.75	-9.37, 5.80
Min, Max	-18.8, 31.3	-50.0, 28.1
Week 52		
n	20	18
Mean (SD)	67.03 (15.46)	73.79 (16.51)
Median	68.75	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 78.13	65.63, 87.50
Min, Max	40.6, 96.9	34.4, 96.9
Change from baseline to Week 52 ^a		
n	17	12
Mean (SD)	0.45 (20.28)	3.13 (12.85)
Median	3.12	3.13
25th, 75th Percentile	-12.50, 9.38	-9.38, 10.94
Min, Max	-43.8, 46.9	-12.5, 28.1
Difference in change from baseline (95%CI)		2.68 (-10.96, 16.32)
P-value ^b		0.6901
Hedges'g (95% CI) ^c		0.15 (-0.59, 0.89)
P-value for interaction term, treatment * [Sex]		0.7545

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported QoLISSY : Social Score		
Baseline		
n	0	2
Mean (SD)		42.19 (11.05)
Median		42.19
25th, 75th Percentile		34.38, 50.00
Min, Max		34.4, 50.0
Week 26		
n	5	7
Mean (SD)	68.13 (18.80)	56.70 (21.46)
Median	68.75	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 81.25	31.25, 78.13
Min, Max	43.8, 90.6	31.3, 81.3
Change from baseline to Week 26 ^a		
n	0	1
Mean (SD)		-3.13 (NA)
Median		-3.13
25th, 75th Percentile		-3.13, -3.13
Min, Max		-3.1, -3.1
Week 52		
n	8	11
Mean (SD)	57.43 (33.24)	62.79 (21.58)
Median	60.94	65.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.69, 84.38	37.50, 84.38
Min, Max	9.4, 100.0	34.4, 93.8
Change from baseline to Week 52 ^a		
n	0	2
Mean (SD)		3.13 (0.01)
Median		3.13
25th, 75th Percentile		3.12, 3.13
Min, Max		3.1, 3.1
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported QoLISSY : Social Score		
Baseline		
n	24	16
Mean (SD)	63.58 (20.31)	66.60 (18.57)
Median	65.63	68.75
25th, 75th Percentile	50.00, 76.79	56.26, 76.57
Min, Max	28.1, 96.9	18.8, 96.9
Week 26		
n	23	14
Mean (SD)	67.43 (21.67)	66.52 (19.16)
Median	65.63	66.75

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 81.25	50.00, 81.25
Min, Max	12.5, 100.0	37.5, 96.9
Change from baseline to Week 26 ^a		
n	23	14
Mean (SD)	4.21 (25.07)	0.67 (13.78)
Median	3.13	1.56
25th, 75th Percentile	-6.25, 21.87	-9.37, 3.13
Min, Max	-84.4, 32.6	-18.8, 28.1
Week 52		
n	24	16
Mean (SD)	60.29 (22.45)	67.78 (17.14)
Median	62.50	67.19

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.69, 70.32	54.69, 79.69
Min, Max	0.0, 96.9	37.5, 96.9
Change from baseline to Week 52 ^a		
n	24	15
Mean (SD)	-3.29 (27.46)	1.67 (16.40)
Median	-0.01	0.00
25th, 75th Percentile	-15.85, 12.50	-9.38, 9.37
Min, Max	-81.3, 46.9	-28.1, 37.5
Difference in change from baseline (95%CI)		4.96 (-9.28, 19.19)
P-value ^b		0.4848
Hedges'g (95% CI) ^c		0.20 (-0.44, 0.85)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported QoLISSY : Social Score		
Baseline		
n	13	12
Mean (SD)	76.21 (18.95)	69.31 (21.20)
Median	78.13	71.88
25th, 75th Percentile	62.50, 90.63	62.50, 85.94
Min, Max	34.4, 100.0	21.9, 96.9
Week 26		
n	12	11
Mean (SD)	78.72 (13.80)	71.84 (15.37)
Median	82.82	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 88.40	65.63, 75.00
Min, Max	53.1, 96.9	46.9, 100.0
Change from baseline to Week 26 ^a		
n	12	11
Mean (SD)	2.16 (10.13)	3.90 (22.81)
Median	-1.56	5.80
25th, 75th Percentile	-3.13, 10.49	-6.69, 25.00
Min, Max	-12.5, 18.8	-50.0, 34.4
Week 52		
n	13	9
Mean (SD)	76.20 (15.07)	75.45 (9.88)
Median	78.13	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	71.88, 87.50	75.00, 81.25
Min, Max	40.6, 96.9	59.4, 87.5
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	0.00 (19.14)	3.57 (17.18)
Median	-3.13	-3.12
25th, 75th Percentile	-9.38, 9.37	-9.37, 9.37
Min, Max	-43.8, 37.5	-9.4, 41.5
Difference in change from baseline (95%CI)		3.57 (-13.05, 20.20)
P-value ^b		0.6587
Hedges'g (95% CI) ^c		0.19 (-0.67, 1.04)
P-value for interaction term, treatment * [Age at Baseline]		0.9080

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.8.2.3.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported QoLISSY : Social Score		
Baseline		
n	24	18
Mean (SD)	68.33 (17.78)	61.31 (19.50)
Median	67.19	62.50
25th, 75th Percentile	60.94, 81.48	50.00, 71.88
Min, Max	34.4, 96.9	18.8, 96.9
Week 26		
n	27	21
Mean (SD)	68.93 (21.10)	63.29 (19.52)
Median	68.75	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 84.38	46.88, 75.00
Min, Max	12.5, 100.0	31.3, 96.9
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	1.12 (24.17)	4.40 (15.59)
Median	0.00	3.12
25th, 75th Percentile	-6.25, 12.50	-6.25, 12.50
Min, Max	-84.4, 32.6	-18.8, 34.4
Week 52		
n	32	26
Mean (SD)	60.45 (25.11)	64.10 (18.66)
Median	62.50	60.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.01, 81.25	50.00, 82.14
Min, Max	0.0, 100.0	34.4, 96.9
Change from baseline to Week 52 ^a		
n	24	16
Mean (SD)	-6.86 (25.55)	3.77 (18.77)
Median	-4.69	0.00
25th, 75th Percentile	-15.85, 10.94	-7.81, 10.94
Min, Max	-81.3, 34.4	-28.1, 41.5
Difference in change from baseline (95%CI)		10.63 (-4.47, 25.73)
P-value ^b		0.1623
Hedges'g (95% CI) ^c		0.45 (-0.19, 1.09)

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.8.2.3.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported QoLISSY : Social Score		
Baseline		
n	13	12
Mean (SD)	67.45 (25.59)	73.18 (19.15)
Median	71.88	75.00
25th, 75th Percentile	50.00, 89.29	67.19, 87.50
Min, Max	28.1, 100.0	21.9, 96.9
Week 26		
n	13	11
Mean (SD)	75.00 (15.78)	71.76 (16.55)
Median	75.00	71.43

Max, maximum; Min, minimum; SD, standard deviation.

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Table 14.2.8.2.3.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 84.38	65.63, 87.50
Min, Max	50.0, 100.0	46.9, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	7.55 (14.08)	-1.54 (20.34)
Median	10.71	3.13
25th, 75th Percentile	-3.13, 18.75	-10.27, 5.80
Min, Max	-12.5, 31.3	-50.0, 25.0
Week 52		
n	13	10
Mean (SD)	74.04 (16.85)	78.75 (6.56)
Median	71.88	76.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 87.50	75.00, 81.25
Min, Max	40.6, 96.9	68.8, 90.6
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	6.59 (20.99)	0.31 (9.37)
Median	0.00	0.00
25th, 75th Percentile	-9.38, 18.75	-9.37, 6.25
Min, Max	-20.5, 46.9	-9.4, 18.8
Difference in change from baseline (95%CI)		-6.28 (-20.03, 7.47)
P-value ^b		0.3495
Hedges'g (95% CI) ^c		-0.36 (-1.18, 0.48)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.1368

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported QoLISSY : Social Score		
Baseline		
n	4	9
Mean (SD)	66.41 (3.93)	65.28 (15.02)
Median	65.63	62.50
25th, 75th Percentile	64.07, 68.76	59.38, 71.88
Min, Max	62.5, 71.9	40.6, 87.5
Week 26		
n	6	8
Mean (SD)	60.42 (15.01)	68.36 (19.94)
Median	59.38	68.76

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 68.75	53.13, 82.82
Min, Max	43.8, 84.4	40.6, 96.9
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-3.91 (13.35)	8.34 (19.63)
Median	-4.69	3.13
25th, 75th Percentile	-14.07, 6.25	0.00, 28.13
Min, Max	-18.8, 12.5	-18.8, 34.4
Week 52		
n	6	10
Mean (SD)	53.13 (22.79)	77.28 (16.57)
Median	62.51	81.70

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 68.75	65.63, 90.63
Min, Max	12.5, 71.9	50.0, 96.9
Change from baseline to Week 52 ^a		
n	4	7
Mean (SD)	-4.69 (13.62)	13.52 (17.88)
Median	1.56	12.50
25th, 75th Percentile	-12.50, 3.13	3.13, 28.13
Min, Max	-25.0, 3.1	-12.5, 41.5
Difference in change from baseline (95%CI)		18.21 (-5.30, 41.72)
P-value ^b		0.1137
Hedges'g (95% CI) ^c		1.00 (-0.33, 2.29)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported QoLISSY : Social Score		
Baseline		
n	15	9
Mean (SD)	71.67 (21.86)	65.98 (18.71)
Median	71.88	65.63
25th, 75th Percentile	59.38, 87.50	53.13, 71.88
Min, Max	31.3, 100.0	34.4, 96.9
Week 26		
n	14	9
Mean (SD)	73.50 (18.41)	61.16 (23.03)
Median	78.13	71.43

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 89.29	37.50, 75.00
Min, Max	37.5, 96.9	31.3, 93.8
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	3.19 (14.11)	-0.34 (8.60)
Median	-3.13	0.00
25th, 75th Percentile	-6.25, 8.48	-4.69, 4.91
Min, Max	-12.5, 31.3	-15.6, 12.5
Week 52		
n	17	12
Mean (SD)	67.10 (22.78)	59.38 (18.89)
Median	71.88	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 84.38	42.19, 75.00
Min, Max	9.4, 96.9	34.4, 90.6
Change from baseline to Week 52 ^a		
n	15	9
Mean (SD)	0.62 (20.16)	-0.35 (7.72)
Median	9.37	-3.12
25th, 75th Percentile	-9.38, 12.50	-6.25, 6.25
Min, Max	-43.8, 37.5	-12.5, 9.4
Difference in change from baseline (95%CI)		-0.97 (-13.10, 11.15)
P-value ^b		0.8687
Hedges'g (95% CI) ^c		-0.06 (-0.88, 0.77)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported QoLISSY : Social Score		
Baseline		
n	12	8
Mean (SD)	64.21 (22.45)	60.22 (28.33)
Median	60.94	70.32
25th, 75th Percentile	50.00, 82.15	34.38, 78.35
Min, Max	28.1, 96.9	18.8, 96.9
Week 26		
n	14	11
Mean (SD)	69.04 (24.00)	65.26 (17.24)
Median	73.44	67.86

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 84.38	46.88, 78.13
Min, Max	12.5, 100.0	46.9, 100.0
Change from baseline to Week 26 ^a		
n	12	8
Mean (SD)	2.01 (28.99)	2.18 (25.84)
Median	10.05	3.12
25th, 75th Percentile	1.56, 18.75	-8.48, 25.00
Min, Max	-84.4, 21.9	-50.0, 28.1
Week 52		
n	16	10
Mean (SD)	64.65 (26.41)	67.50 (15.25)
Median	67.19	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 84.38	56.25, 78.13
Min, Max	0.0, 100.0	37.5, 87.5
Change from baseline to Week 52 ^a		
n	12	6
Mean (SD)	-5.62 (35.65)	-0.52 (21.96)
Median	-6.25	-4.69
25th, 75th Percentile	-18.08, 20.31	-9.38, 6.25
Min, Max	-81.3, 46.9	-28.1, 37.5
Difference in change from baseline (95%CI)		5.10 (-28.83, 39.02)
P-value ^b		0.7543
Hedges'g (95% CI) ^c		0.15 (-0.83, 1.13)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported QoLISSY : Social Score		
Baseline		
n	6	4
Mean (SD)	67.56 (22.23)	79.69 (9.38)
Median	71.43	81.25
25th, 75th Percentile	50.00, 87.50	71.88, 87.50
Min, Max	34.4, 90.6	68.8, 87.5
Week 26		
n	6	4
Mean (SD)	79.69 (10.96)	75.78 (8.97)
Median	76.57	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	75.00, 87.50	70.32, 81.25
Min, Max	65.6, 96.9	65.6, 87.5
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	12.13 (20.62)	-3.91 (8.60)
Median	12.50	-4.69
25th, 75th Percentile	-3.57, 31.25	-10.94, 3.13
Min, Max	-12.5, 32.6	-12.5, 6.3
Week 52		
n	6	4
Mean (SD)	67.19 (21.72)	73.44 (9.71)
Median	67.19	76.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 87.50	67.19, 79.69
Min, Max	31.3, 90.6	59.4, 81.3
Change from baseline to Week 52 ^a		
n	6	4
Mean (SD)	-0.37 (16.14)	-6.25 (4.42)
Median	-3.13	-7.81
25th, 75th Percentile	-15.62, 9.38	-9.37, -3.13
Min, Max	-16.1, 26.3	-9.4, 0.0
Difference in change from baseline (95%CI)		-5.88 (-25.29, 13.54)
P-value ^b		0.5051
Hedges'g (95% CI) ^c		-0.41 (-1.67, 0.89)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.6090

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported QoLISSY : Social Score		
Baseline		
n	13	10
Mean (SD)	69.27 (19.06)	72.86 (14.00)
Median	65.63	70.32
25th, 75th Percentile	59.38, 78.57	62.50, 84.38
Min, Max	28.1, 96.9	50.0, 96.9
Week 26		
n	14	10
Mean (SD)	65.85 (18.81)	72.50 (13.16)
Median	68.75	73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 81.25	68.75, 81.25
Min, Max	12.5, 84.4	43.8, 93.8
Change from baseline to Week 26 ^a		
n	13	7
Mean (SD)	-3.64 (26.97)	0.83 (7.00)
Median	-3.13	0.00
25th, 75th Percentile	-6.25, 12.50	-6.25, 6.25
Min, Max	-84.4, 21.9	-6.7, 12.5
Week 52		
n	15	10
Mean (SD)	57.71 (27.82)	66.57 (18.58)
Median	62.50	59.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.63, 71.88	53.13, 84.38
Min, Max	9.4, 96.9	37.5, 90.6
Change from baseline to Week 52 ^a		
n	13	8
Mean (SD)	-4.36 (31.09)	-8.98 (9.37)
Median	0.00	-7.81
25th, 75th Percentile	-16.07, 9.37	-12.50, -3.12
Min, Max	-81.3, 46.9	-28.1, 3.1
Difference in change from baseline (95%CI)		-4.62 (-24.29, 15.04)
P-value ^b		0.6241
Hedges'g (95% CI) ^c		-0.17 (-1.06, 0.71)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported QoLISSY : Social Score		
Baseline		
n	14	9
Mean (SD)	69.42 (23.77)	55.56 (23.22)
Median	73.44	53.13
25th, 75th Percentile	50.00, 87.50	40.63, 68.75
Min, Max	31.3, 100.0	18.8, 96.9
Week 26		
n	13	12
Mean (SD)	75.55 (16.01)	55.55 (20.44)
Median	78.13	52.01

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.50, 87.50	39.07, 70.32
Min, Max	46.9, 96.9	31.3, 96.9
Change from baseline to Week 26 ^a		
n	12	9
Mean (SD)	8.15 (14.25)	-0.25 (27.32)
Median	8.93	-3.13
25th, 75th Percentile	-3.13, 18.75	-15.63, 28.13
Min, Max	-12.5, 31.3	-50.0, 34.4
Week 52		
n	15	12
Mean (SD)	68.54 (16.64)	65.70 (18.82)
Median	71.88	67.19

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 84.38	51.57, 78.57
Min, Max	40.6, 87.5	37.5, 96.9
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	0.67 (20.66)	12.94 (18.69)
Median	-3.13	9.37
25th, 75th Percentile	-9.38, 9.38	0.00, 28.13
Min, Max	-43.8, 37.5	-9.4, 41.5
Difference in change from baseline (95%CI)		12.28 (-5.44, 29.99)
P-value ^b		0.1642
Hedges'g (95% CI) ^c		0.59 (-0.27, 1.44)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.003.006_qs_sum_ovr_qol_self_soc_bhagv_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported QoLISSY : Social Score		
Baseline		
n	10	11
Mean (SD)	64.42 (19.01)	68.47 (19.79)
Median	64.96	71.88
25th, 75th Percentile	50.00, 68.75	62.50, 87.50
Min, Max	34.4, 96.9	21.9, 87.5
Week 26		
n	13	10
Mean (SD)	71.71 (23.39)	72.68 (16.83)
Median	65.63	71.66

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 96.88	65.63, 87.50
Min, Max	37.5, 100.0	46.9, 100.0
Change from baseline to Week 26 ^a		
n	10	10
Mean (SD)	7.23 (18.16)	4.55 (12.37)
Median	3.13	3.13
25th, 75th Percentile	-6.25, 28.13	0.00, 5.80
Min, Max	-18.8, 32.6	-12.5, 25.0
Week 52		
n	15	14
Mean (SD)	66.88 (25.25)	71.43 (16.16)
Median	68.75	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.3.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 84.38	65.63, 81.25
Min, Max	0.0, 100.0	34.4, 93.8
Change from baseline to Week 52 ^a		
n	10	9
Mean (SD)	-3.17 (22.34)	2.08 (9.37)
Median	-0.01	3.13
25th, 75th Percentile	-15.62, 12.50	-6.25, 6.25
Min, Max	-50.0, 26.3	-9.4, 18.8
Difference in change from baseline (95%CI)		5.25 (-11.53, 22.03)
P-value ^b		0.5090
Hedges'g (95% CI) ^c		0.29 (-0.62, 1.19)
P-value for interaction term, treatment * [Baseline AGV]		0.4433

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported QoLISSY : Social Score		
Baseline		
n	26	24
Mean (SD)	66.30 (20.62)	66.04 (19.35)
Median	65.63	65.63
25th, 75th Percentile	50.00, 84.38	53.13, 81.48
Min, Max	28.1, 100.0	21.9, 96.9
Week 26		
n	28	27
Mean (SD)	71.05 (19.69)	63.64 (17.15)
Median	73.44	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.3.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	58.26, 84.38	46.88, 75.00
Min, Max	12.5, 96.9	31.3, 93.8
Change from baseline to Week 26 ^a		
n	25	21
Mean (SD)	3.87 (23.52)	-1.23 (16.61)
Median	3.13	0.00
25th, 75th Percentile	-3.13, 18.75	-6.69, 4.01
Min, Max	-84.4, 32.6	-50.0, 34.4
Week 52		
n	31	27
Mean (SD)	63.71 (26.22)	67.28 (16.32)
Median	68.75	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.3.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 84.38	53.13, 81.25
Min, Max	0.0, 100.0	37.5, 90.6
Change from baseline to Week 52 ^a		
n	26	21
Mean (SD)	-1.51 (27.84)	-0.26 (14.31)
Median	1.56	-3.12
25th, 75th Percentile	-9.38, 12.50	-9.37, 6.25
Min, Max	-81.3, 46.9	-28.1, 41.5
Difference in change from baseline (95%CI)		1.26 (-11.47, 13.98)
P-value ^b		0.8428
Hedges'g (95% CI) ^c		0.05 (-0.52, 0.63)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.3.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported QoLISSY : Social Score		
Baseline		
n	11	6
Mean (SD)	72.08 (20.60)	66.15 (24.08)
Median	78.13	73.44
25th, 75th Percentile	59.38, 89.29	68.75, 75.00
Min, Max	31.3, 96.9	18.8, 87.5
Week 26		
n	12	5
Mean (SD)	70.57 (20.05)	80.00 (22.92)
Median	75.00	90.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.3.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 82.82	65.63, 96.88
Min, Max	37.5, 100.0	46.9, 100.0
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	2.59 (13.75)	15.00 (17.17)
Median	-3.13	25.00
25th, 75th Percentile	-6.25, 10.71	3.13, 28.13
Min, Max	-12.5, 31.3	-9.4, 28.1
Week 52		
n	14	9
Mean (SD)	65.85 (17.53)	70.84 (21.31)
Median	62.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.3.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Social Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 84.38	56.25, 90.63
Min, Max	40.6, 96.9	34.4, 96.9
Change from baseline to Week 52 ^a		
n	11	5
Mean (SD)	-3.61 (15.56)	13.75 (17.76)
Median	-3.13	3.13
25th, 75th Percentile	-16.07, 12.50	0.00, 28.13
Min, Max	-25.0, 18.8	0.0, 37.5
Difference in change from baseline (95%CI)		17.36 (-1.40, 36.13)
P-value ^b		0.0671
Hedges'g (95% CI) ^c		1.01 (-0.12, 2.12)
P-value for interaction term, treatment * [Ethnicity]		0.2262

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	20	16
Mean (SD)	73.60 (16.27)	67.39 (25.54)
Median	75.00	70.32
25th, 75th Percentile	67.19, 87.50	45.32, 90.63
Min, Max	40.6, 100.0	21.9, 100.0
Week 26		
n	19	16
Mean (SD)	74.67 (20.93)	71.49 (21.44)
Median	78.13	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 87.50	56.25, 90.63
Min, Max	9.4, 100.0	34.4, 100.0
Change from baseline to Week 26 ^a		
n	17	15
Mean (SD)	-1.47 (19.46)	6.04 (18.95)
Median	3.12	0.00
25th, 75th Percentile	-9.37, 6.25	-3.13, 18.75
Min, Max	-68.8, 18.8	-21.9, 43.8
Week 52		
n	24	19
Mean (SD)	73.44 (23.60)	66.76 (20.38)
Median	76.57	65.63

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 90.63	50.00, 84.38
Min, Max	9.4, 100.0	31.3, 100.0
Change from baseline to Week 52 ^a		
n	19	15
Mean (SD)	1.97 (20.86)	1.43 (24.98)
Median	0.00	-6.25
25th, 75th Percentile	-9.38, 12.50	-18.75, 25.00
Min, Max	-43.8, 59.4	-37.5, 46.9
Difference in change from baseline (95%CI)		-0.55 (-16.56, 15.46)
P-value ^b		0.9451
Hedges'g (95% CI) ^c		-0.02 (-0.70, 0.65)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	16	14
Mean (SD)	66.02 (19.99)	75.90 (15.67)
Median	75.00	79.69
25th, 75th Percentile	54.69, 76.57	65.63, 87.50
Min, Max	6.3, 84.4	40.6, 96.9
Week 26		
n	20	17
Mean (SD)	70.54 (15.07)	78.10 (12.61)
Median	75.00	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	63.17, 79.69	71.88, 84.38
Min, Max	28.1, 93.8	46.9, 100.0
Change from baseline to Week 26 ^a		
n	16	12
Mean (SD)	4.77 (12.50)	1.79 (14.82)
Median	7.81	-1.57
25th, 75th Percentile	-4.69, 12.50	-10.94, 14.06
Min, Max	-23.7, 25.0	-15.6, 25.0
Week 52		
n	20	18
Mean (SD)	63.28 (20.65)	77.95 (19.84)
Median	68.76	84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.69, 78.13	71.88, 93.75
Min, Max	6.3, 87.5	18.8, 96.9
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	-0.59 (22.42)	4.17 (11.18)
Median	0.00	6.25
25th, 75th Percentile	-6.25, 9.38	-4.69, 12.50
Min, Max	-56.3, 37.5	-15.6, 18.8
Difference in change from baseline (95%CI)		4.75 (-8.63, 18.13)
P-value ^b		0.4700
Hedges'g (95% CI) ^c		0.25 (-0.50, 1.00)
P-value for interaction term, treatment * [Sex]		0.6254

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.004.001_qs_sum_ovr_qol_self_emo_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	0	2
Mean (SD)		51.57 (19.89)
Median		51.57
25th, 75th Percentile		37.50, 65.63
Min, Max		37.5, 65.6
Week 26		
n	5	7
Mean (SD)	70.00 (17.48)	70.09 (19.59)
Median	71.88	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 78.13	46.88, 84.38
Min, Max	50.0, 93.8	43.8, 96.9
Change from baseline to Week 26 ^a		
n	0	1
Mean (SD)		6.25 (NA)
Median		6.25
25th, 75th Percentile		6.25, 6.25
Min, Max		6.3, 6.3
Week 52		
n	8	11
Mean (SD)	58.60 (28.14)	65.91 (22.81)
Median	60.94	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.19, 79.69	46.88, 84.38
Min, Max	9.4, 93.8	18.8, 93.8
Change from baseline to Week 52 ^a		
n	0	2
Mean (SD)		21.88 (4.42)
Median		21.88
25th, 75th Percentile		18.75, 25.00
Min, Max		18.8, 25.0
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	23	16
Mean (SD)	68.34 (19.32)	72.27 (23.60)
Median	75.00	78.13
25th, 75th Percentile	56.25, 78.13	64.07, 89.07
Min, Max	6.3, 93.8	21.9, 100.0
Week 26		
n	23	15
Mean (SD)	71.39 (21.24)	74.56 (19.87)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	60.71, 84.38	65.63, 90.63
Min, Max	9.4, 100.0	34.4, 100.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	1.91 (19.51)	2.26 (18.48)
Median	6.25	-3.12
25th, 75th Percentile	-6.25, 9.38	-15.62, 18.75
Min, Max	-68.8, 25.0	-21.9, 43.8
Week 52		
n	23	16
Mean (SD)	66.31 (23.23)	74.97 (19.70)
Median	75.00	82.82

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 81.25	65.63, 89.07
Min, Max	6.3, 96.9	31.3, 93.8
Change from baseline to Week 52 ^a		
n	22	15
Mean (SD)	-2.27 (21.89)	2.05 (19.48)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 9.38	-6.70, 15.62
Min, Max	-56.3, 37.5	-37.5, 43.8
Difference in change from baseline (95%CI)		4.33 (-9.92, 18.57)
P-value ^b		0.5416
Hedges'g (95% CI) ^c		0.20 (-0.46, 0.86)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	13	12
Mean (SD)	73.56 (16.07)	73.44 (18.87)
Median	75.00	73.44
25th, 75th Percentile	75.00, 81.25	59.38, 89.07
Min, Max	40.6, 100.0	40.6, 100.0
Week 26		
n	11	11
Mean (SD)	76.14 (10.10)	78.41 (12.92)
Median	78.13	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 81.25	65.63, 90.63
Min, Max	65.6, 100.0	62.5, 100.0
Change from baseline to Week 26 ^a		
n	11	11
Mean (SD)	0.85 (8.51)	6.53 (16.33)
Median	0.00	3.12
25th, 75th Percentile	-9.37, 6.25	-3.13, 15.62
Min, Max	-9.4, 15.6	-15.6, 43.8
Week 52		
n	13	10
Mean (SD)	79.57 (13.36)	74.69 (20.22)
Median	78.13	73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	71.88, 90.63	59.38, 96.88
Min, Max	53.1, 100.0	40.6, 100.0
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	6.01 (20.03)	-0.31 (21.06)
Median	0.00	-7.81
25th, 75th Percentile	-3.12, 3.13	-15.63, 12.50
Min, Max	-21.9, 59.4	-21.9, 46.9
Difference in change from baseline (95%CI)		-6.32 (-24.24, 11.59)
P-value ^b		0.4709
Hedges'g (95% CI) ^c		-0.30 (-1.12, 0.54)
P-value for interaction term, treatment * [Age at Baseline]		0.3404

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	23	18
Mean (SD)	70.93 (14.61)	66.50 (23.48)
Median	75.00	68.76
25th, 75th Percentile	65.63, 78.13	53.13, 87.50
Min, Max	40.6, 93.8	21.9, 100.0
Week 26		
n	26	22
Mean (SD)	72.41 (18.94)	72.43 (20.11)
Median	75.00	73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 84.38	62.50, 90.63
Min, Max	9.4, 100.0	34.4, 100.0
Change from baseline to Week 26 ^a		
n	20	16
Mean (SD)	0.07 (19.26)	6.61 (19.77)
Median	6.25	1.56
25th, 75th Percentile	-4.69, 9.38	-6.47, 20.31
Min, Max	-68.8, 18.8	-21.9, 43.8
Week 52		
n	31	27
Mean (SD)	68.45 (21.69)	68.98 (21.84)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 81.25	50.00, 84.38
Min, Max	9.4, 100.0	18.8, 100.0
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	0.85 (22.06)	5.51 (23.35)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 12.50	-6.25, 18.75
Min, Max	-43.8, 59.4	-37.5, 46.9
Difference in change from baseline (95%CI)		4.66 (-10.14, 19.47)
P-value ^b		0.5273
Hedges'g (95% CI) ^c		0.20 (-0.43, 0.84)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	13	12
Mean (SD)	68.99 (23.81)	78.65 (16.74)
Median	75.00	81.26
25th, 75th Percentile	56.25, 81.25	70.32, 89.07
Min, Max	6.3, 100.0	40.6, 100.0
Week 26		
n	13	11
Mean (SD)	72.84 (16.85)	79.83 (9.52)
Median	78.13	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.4.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 81.25	75.00, 87.50
Min, Max	28.1, 100.0	65.6, 100.0
Change from baseline to Week 26 ^a		
n	13	11
Mean (SD)	3.85 (11.42)	0.57 (12.09)
Median	3.12	0.00
25th, 75th Percentile	-6.25, 9.37	-6.25, 6.25
Min, Max	-9.4, 25.0	-15.6, 25.0
Week 52		
n	13	10
Mean (SD)	69.71 (25.69)	80.90 (14.50)
Median	75.00	85.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 84.38	71.43, 93.75
Min, Max	6.3, 100.0	56.3, 96.9
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	0.72 (20.85)	-2.23 (10.85)
Median	0.00	-4.91
25th, 75th Percentile	-3.12, 9.37	-12.50, 9.38
Min, Max	-56.3, 37.5	-15.6, 12.5
Difference in change from baseline (95%CI)		-2.96 (-18.08, 12.17)
P-value ^b		0.6886
Hedges'g (95% CI) ^c		-0.16 (-0.99, 0.66)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.4936

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.004.003_qs_sum_ovr_qol_self_emo_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	4	9
Mean (SD)	69.53 (14.52)	71.88 (14.32)
Median	71.88	71.88
25th, 75th Percentile	59.38, 79.69	59.38, 87.50
Min, Max	50.0, 84.4	53.1, 90.6
Week 26		
n	6	8
Mean (SD)	65.85 (9.32)	78.85 (16.88)
Median	63.17	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 71.88	71.66, 92.19
Min, Max	56.3, 81.3	46.9, 100.0
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-2.79 (17.32)	15.55 (17.33)
Median	-1.56	14.06
25th, 75th Percentile	-16.52, 10.94	0.00, 25.00
Min, Max	-23.7, 15.6	-3.6, 43.8
Week 52		
n	6	10
Mean (SD)	62.50 (12.50)	85.00 (10.18)
Median	65.63	84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 71.88	84.38, 93.75
Min, Max	40.6, 75.0	65.6, 100.0
Change from baseline to Week 52 ^a		
n	4	7
Mean (SD)	-2.34 (21.71)	14.73 (16.51)
Median	-3.12	12.50
25th, 75th Percentile	-15.63, 10.94	0.00, 18.75
Min, Max	-28.1, 25.0	-3.1, 46.9
Difference in change from baseline (95%CI)		17.08 (-9.02, 43.17)
P-value ^b		0.1729
Hedges'g (95% CI) ^c		0.85 (-0.46, 2.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	14	9
Mean (SD)	75.23 (16.37)	70.49 (22.81)
Median	76.57	78.13
25th, 75th Percentile	65.63, 87.50	62.50, 84.38
Min, Max	40.6, 100.0	31.3, 96.9
Week 26		
n	13	10
Mean (SD)	78.13 (13.07)	71.25 (17.36)
Median	78.13	70.32

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	75.00, 81.25	62.50, 81.25
Min, Max	50.0, 100.0	43.8, 100.0
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	1.14 (11.03)	1.39 (20.68)
Median	0.00	0.00
25th, 75th Percentile	-9.37, 3.12	-15.62, 6.25
Min, Max	-12.5, 25.0	-21.9, 43.8
Week 52		
n	17	12
Mean (SD)	74.63 (22.37)	64.59 (24.87)
Median	78.13	60.94

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	71.88, 87.50	48.44, 89.07
Min, Max	9.4, 100.0	18.8, 96.9
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	6.03 (19.32)	2.78 (20.28)
Median	3.13	0.00
25th, 75th Percentile	-3.12, 12.50	-6.25, 15.62
Min, Max	-21.9, 59.4	-37.5, 28.1
Difference in change from baseline (95%CI)		-3.25 (-20.74, 14.25)
P-value ^b		0.7032
Hedges'g (95% CI) ^c		-0.16 (-1.00, 0.68)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	12	8
Mean (SD)	63.80 (21.38)	66.41 (29.59)
Median	75.00	73.44
25th, 75th Percentile	54.69, 75.00	39.07, 92.19
Min, Max	6.3, 84.4	21.9, 100.0
Week 26		
n	14	11
Mean (SD)	68.98 (23.65)	74.15 (20.11)
Median	78.13	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 84.38	65.63, 90.63
Min, Max	9.4, 93.8	34.4, 96.9
Change from baseline to Week 26 ^a		
n	12	8
Mean (SD)	2.35 (23.31)	2.74 (13.72)
Median	9.38	0.01
25th, 75th Percentile	4.69, 10.94	-6.25, 12.50
Min, Max	-68.8, 21.9	-15.6, 25.0
Week 52		
n	16	11
Mean (SD)	64.85 (26.16)	69.28 (18.37)
Median	70.32	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 82.82	56.25, 81.25
Min, Max	6.3, 96.9	31.3, 96.9
Change from baseline to Week 52 ^a		
n	12	7
Mean (SD)	-3.12 (27.11)	-1.85 (22.54)
Median	0.00	-6.70
25th, 75th Percentile	-14.06, 12.51	-18.75, 9.38
Min, Max	-56.3, 37.5	-21.9, 43.8
Difference in change from baseline (95%CI)		1.27 (-24.41, 26.95)
P-value ^b		0.9179
Hedges'g (95% CI) ^c		0.05 (-0.89, 0.98)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	6	4
Mean (SD)	71.88 (17.45)	82.04 (17.56)
Median	75.00	84.38
25th, 75th Percentile	68.75, 78.13	68.76, 95.32
Min, Max	40.6, 93.8	59.4, 100.0
Week 26		
n	6	4
Mean (SD)	75.52 (18.48)	78.13 (15.73)
Median	76.57	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 87.50	68.75, 87.50
Min, Max	46.9, 100.0	62.5, 100.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	3.65 (10.16)	-3.91 (8.22)
Median	6.25	-1.57
25th, 75th Percentile	-6.25, 6.25	-9.38, 1.56
Min, Max	-9.4, 18.8	-15.6, 3.1
Week 52		
n	5	4
Mean (SD)	69.38 (21.92)	71.10 (24.39)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	75.00, 78.13	51.57, 90.63
Min, Max	31.3, 87.5	40.6, 93.8
Change from baseline to Week 52 ^a		
n	5	4
Mean (SD)	-1.88 (10.50)	-10.94 (9.71)
Median	0.00	-14.07
25th, 75th Percentile	-9.38, 6.25	-17.19, -4.69
Min, Max	-15.6, 9.4	-18.8, 3.1
Difference in change from baseline (95%CI)		-9.06 (-25.20, 7.07)
P-value ^b		0.2258
Hedges'g (95% CI) ^c		-0.79 (-2.14, 0.61)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.5179

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	13	10
Mean (SD)	67.79 (22.37)	78.13 (16.27)
Median	75.00	79.69
25th, 75th Percentile	68.75, 78.13	62.50, 90.63
Min, Max	6.3, 90.6	56.3, 100.0
Week 26		
n	14	11
Mean (SD)	67.86 (23.49)	75.85 (15.57)
Median	76.57	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 81.25	62.50, 90.63
Min, Max	9.4, 93.8	46.9, 100.0
Change from baseline to Week 26 ^a		
n	13	8
Mean (SD)	-0.24 (22.77)	1.17 (15.48)
Median	3.12	0.00
25th, 75th Percentile	-3.12, 9.38	-12.50, 12.50
Min, Max	-68.8, 21.9	-15.6, 25.0
Week 52		
n	15	11
Mean (SD)	65.42 (26.82)	75.29 (15.27)
Median	75.00	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 81.25	65.63, 84.38
Min, Max	6.3, 90.6	40.6, 93.8
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	1.92 (18.77)	-2.78 (15.19)
Median	0.00	-3.12
25th, 75th Percentile	-3.12, 6.25	-18.75, 9.38
Min, Max	-43.8, 37.5	-21.9, 18.8
Difference in change from baseline (95%CI)		-4.70 (-20.46, 11.06)
P-value ^b		0.5409
Hedges'g (95% CI) ^c		-0.26 (-1.11, 0.60)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	14	9
Mean (SD)	70.54 (15.10)	61.81 (26.51)
Median	75.00	71.88
25th, 75th Percentile	56.25, 75.00	37.50, 87.50
Min, Max	40.6, 100.0	21.9, 90.6
Week 26		
n	12	12
Mean (SD)	73.70 (12.32)	74.18 (20.87)
Median	73.44	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 81.25	57.82, 93.76
Min, Max	50.0, 100.0	40.6, 100.0
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	3.69 (10.53)	9.67 (23.13)
Median	3.13	6.25
25th, 75th Percentile	-6.25, 9.38	-3.57, 18.75
Min, Max	-9.4, 25.0	-21.9, 43.8
Week 52		
n	14	12
Mean (SD)	75.00 (20.25)	72.40 (22.64)
Median	75.00	73.44

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 90.63	53.13, 93.75
Min, Max	37.5, 100.0	34.4, 100.0
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	7.21 (20.94)	15.28 (25.70)
Median	3.13	18.75
25th, 75th Percentile	0.00, 18.75	3.12, 28.13
Min, Max	-21.9, 59.4	-37.5, 46.9
Difference in change from baseline (95%CI)		8.07 (-12.70, 28.83)
P-value ^b		0.4274
Hedges'g (95% CI) ^c		0.34 (-0.52, 1.19)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	9	11
Mean (SD)	73.27 (17.34)	73.01 (20.50)
Median	75.00	78.13
25th, 75th Percentile	65.63, 84.38	59.38, 87.50
Min, Max	40.6, 93.8	37.5, 100.0
Week 26		
n	13	10
Mean (SD)	76.55 (15.80)	74.69 (16.89)
Median	78.13	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 87.50	75.00, 81.25
Min, Max	46.9, 100.0	34.4, 100.0
Change from baseline to Week 26 ^a		
n	9	10
Mean (SD)	1.54 (12.75)	1.56 (11.53)
Median	6.25	-1.56
25th, 75th Percentile	-6.25, 9.38	-3.13, 6.25
Min, Max	-23.7, 12.5	-15.6, 25.0
Week 52		
n	15	14
Mean (SD)	66.46 (20.44)	69.61 (23.46)
Median	75.00	75.01

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 78.13	56.25, 87.50
Min, Max	28.1, 93.8	18.8, 96.9
Change from baseline to Week 52 ^a		
n	9	9
Mean (SD)	-10.07 (23.27)	-4.56 (10.39)
Median	-9.38	-6.70
25th, 75th Percentile	-15.62, 0.00	-12.50, -3.12
Min, Max	-56.3, 28.1	-15.6, 12.5
Difference in change from baseline (95%CI)		5.51 (-13.18, 24.19)
P-value ^b		0.5301
Hedges'g (95% CI) ^c		0.29 (-0.64, 1.22)
P-value for interaction term, treatment * [Baseline AGV]		0.5438

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	25	24
Mean (SD)	68.63 (20.97)	71.88 (21.28)
Median	75.00	78.13
25th, 75th Percentile	53.13, 81.25	57.82, 89.07
Min, Max	6.3, 100.0	31.3, 100.0
Week 26		
n	27	28
Mean (SD)	72.51 (20.29)	75.78 (17.67)
Median	75.00	76.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 84.38	65.63, 90.63
Min, Max	9.4, 100.0	34.4, 100.0
Change from baseline to Week 26 ^a		
n	23	22
Mean (SD)	1.42 (18.08)	4.83 (18.09)
Median	6.25	0.00
25th, 75th Percentile	0.00, 9.38	-6.25, 15.62
Min, Max	-68.8, 21.9	-21.9, 43.8
Week 52		
n	30	28
Mean (SD)	69.79 (24.80)	72.53 (19.23)
Median	75.00	76.57

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	56.25, 87.50	60.94, 87.50
Min, Max	6.3, 100.0	31.3, 100.0
Change from baseline to Week 52 ^a		
n	24	22
Mean (SD)	2.34 (22.06)	0.97 (19.38)
Median	0.00	-1.56
25th, 75th Percentile	-9.38, 15.63	-12.50, 12.50
Min, Max	-43.8, 59.4	-37.5, 46.9
Difference in change from baseline (95%CI)		-1.37 (-13.76, 11.02)
P-value ^b		0.8246
Hedges'g (95% CI) ^c		-0.06 (-0.64, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported QoLISSY : Emotional Score		
Baseline		
n	11	6
Mean (SD)	73.87 (8.76)	69.27 (24.80)
Median	75.00	73.44
25th, 75th Percentile	68.75, 78.13	68.75, 87.50
Min, Max	56.3, 87.5	21.9, 90.6
Week 26		
n	12	5
Mean (SD)	72.66 (12.29)	69.91 (17.47)
Median	76.57	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.4.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	62.51, 81.25	71.43, 75.00
Min, Max	50.0, 87.5	40.6, 87.5
Change from baseline to Week 26 ^a		
n	10	5
Mean (SD)	1.88 (13.03)	1.16 (12.66)
Median	-1.57	0.00
25th, 75th Percentile	-9.37, 12.50	-3.57, 6.25
Min, Max	-12.5, 25.0	-15.6, 18.8
Week 52		
n	14	9
Mean (SD)	66.74 (17.79)	71.18 (25.81)
Median	70.32	84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.4.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Emotional Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	65.63, 78.13	56.25, 93.75
Min, Max	28.1, 87.5	18.8, 93.8
Change from baseline to Week 52 ^a		
n	11	5
Mean (SD)	-2.56 (20.15)	10.00 (22.03)
Median	3.12	3.12
25th, 75th Percentile	-9.37, 9.37	-3.12, 18.75
Min, Max	-56.3, 21.9	-12.5, 43.8
Difference in change from baseline (95%CI)		12.56 (-11.39, 36.50)
P-value ^b		0.2797
Hedges'g (95% CI) ^c		0.57 (-0.51, 1.64)
P-value for interaction term, treatment * [Ethnicity]		0.2805

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	20	16
Mean (SD)	45.53 (22.71)	58.28 (24.91)
Median	45.00	60.00
25th, 75th Percentile	27.50, 65.32	36.25, 82.50
Min, Max	2.5, 77.5	20.0, 92.5
Week 26		
n	20	16
Mean (SD)	38.75 (23.40)	56.32 (23.71)
Median	33.75	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 48.75	38.75, 74.59
Min, Max	0.0, 95.0	20.0, 95.0
Change from baseline to Week 26 ^a		
n	18	15
Mean (SD)	-4.48 (27.07)	-0.26 (18.44)
Median	-2.50	-5.00
25th, 75th Percentile	-15.00, 15.00	-10.00, 7.50
Min, Max	-65.0, 40.0	-35.0, 32.5
Week 52		
n	25	19
Mean (SD)	49.31 (24.57)	51.01 (21.91)
Median	47.50	45.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 62.50	32.50, 62.50
Min, Max	15.0, 100.0	20.0, 92.5
Change from baseline to Week 52 ^a		
n	20	15
Mean (SD)	0.11 (23.12)	-4.72 (19.45)
Median	1.25	-2.50
25th, 75th Percentile	-10.00, 13.75	-22.50, 5.00
Min, Max	-50.0, 42.5	-42.5, 30.0
Difference in change from baseline (95%CI)		-4.83 (-19.87, 10.21)
P-value ^b		0.5180
Hedges'g (95% CI) ^c		-0.22 (-0.89, 0.46)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	16	14
Mean (SD)	50.89 (17.60)	42.14 (19.53)
Median	46.25	38.75
25th, 75th Percentile	42.09, 60.00	30.00, 57.50
Min, Max	22.5, 100.0	5.0, 72.5
Week 26		
n	19	17
Mean (SD)	52.27 (16.29)	44.71 (18.60)
Median	55.56	45.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.50, 62.50	27.50, 62.50
Min, Max	22.5, 75.0	15.0, 72.5
Change from baseline to Week 26 ^a		
n	15	12
Mean (SD)	3.76 (24.68)	1.25 (16.70)
Median	7.50	2.50
25th, 75th Percentile	-6.67, 25.00	-6.25, 10.00
Min, Max	-46.9, 37.5	-32.5, 32.5
Week 52		
n	20	18
Mean (SD)	47.03 (17.57)	47.30 (25.01)
Median	47.50	47.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 56.25	27.50, 63.89
Min, Max	10.0, 82.5	7.5, 100.0
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	-5.22 (24.48)	1.57 (24.32)
Median	-7.50	-1.25
25th, 75th Percentile	-19.69, 7.36	-17.50, 20.00
Min, Max	-50.0, 60.0	-32.5, 42.5
Difference in change from baseline (95%CI)		6.79 (-12.37, 25.96)
P-value ^b		0.4726
Hedges'g (95% CI) ^c		0.27 (-0.48, 1.02)
P-value for interaction term, treatment *[Sex]		0.3259

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	0	2
Mean (SD)		66.25 (12.37)
Median		66.25
25th, 75th Percentile		57.50, 75.00
Min, Max		57.5, 75.0
Week 26		
n	5	7
Mean (SD)	38.00 (12.55)	52.50 (17.38)
Median	45.00	57.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 45.00	30.00, 65.00
Min, Max	20.0, 50.0	27.5, 72.5
Change from baseline to Week 26 ^a		
n	0	1
Mean (SD)		-10.00 (NA)
Median		-10.00
25th, 75th Percentile		-10.00, -10.00
Min, Max		-10.0, -10.0
Week 52		
n	8	11
Mean (SD)	57.19 (20.81)	56.59 (14.80)
Median	50.00	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 72.50	40.00, 67.50
Min, Max	35.0, 90.0	30.0, 77.5
Change from baseline to Week 52 ^a		
n	0	2
Mean (SD)		10.00 (10.61)
Median		10.00
25th, 75th Percentile		2.50, 17.50
Min, Max		2.5, 17.5
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	23	16
Mean (SD)	50.75 (20.94)	47.50 (24.51)
Median	45.00	46.25
25th, 75th Percentile	35.00, 69.44	31.25, 62.50
Min, Max	17.5, 100.0	5.0, 90.0
Week 26		
n	23	15
Mean (SD)	45.57 (20.90)	44.13 (19.26)
Median	42.50	45.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 57.50	27.50, 55.00
Min, Max	10.0, 95.0	15.0, 85.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	-4.74 (23.31)	-4.37 (17.28)
Median	-3.75	-2.50
25th, 75th Percentile	-15.00, 15.00	-20.00, 10.00
Min, Max	-52.5, 30.6	-35.0, 26.9
Week 52		
n	24	16
Mean (SD)	47.85 (21.15)	46.49 (24.78)
Median	43.75	40.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	36.25, 62.50	30.00, 63.20
Min, Max	15.0, 100.0	7.5, 100.0
Change from baseline to Week 52 ^a		
n	23	15
Mean (SD)	-3.97 (21.61)	-1.41 (22.63)
Median	0.00	-7.50
25th, 75th Percentile	-17.50, 10.00	-22.50, 22.50
Min, Max	-50.0, 42.5	-30.0, 42.5
Difference in change from baseline (95%CI)		2.57 (-12.25, 17.38)
P-value ^b		0.7274
Hedges'g (95% CI) ^c		0.11 (-0.54, 0.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	13	12
Mean (SD)	42.88 (19.44)	52.50 (24.05)
Median	47.50	48.75
25th, 75th Percentile	27.50, 60.00	33.75, 71.25
Min, Max	2.5, 65.0	22.5, 92.5
Week 26		
n	11	11
Mean (SD)	48.18 (25.23)	57.42 (26.29)
Median	55.00	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.50, 72.50	27.50, 82.50
Min, Max	0.0, 80.0	20.0, 95.0
Change from baseline to Week 26 ^a		
n	11	11
Mean (SD)	7.27 (30.11)	7.88 (16.12)
Median	10.00	2.50
25th, 75th Percentile	0.00, 37.50	-5.00, 29.17
Min, Max	-65.0, 40.0	-12.5, 32.5
Week 52		
n	13	10
Mean (SD)	43.65 (22.68)	45.42 (28.17)
Median	47.50	44.59

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 55.00	22.50, 62.50
Min, Max	10.0, 82.5	7.5, 92.5
Change from baseline to Week 52 ^a		
n	13	10
Mean (SD)	0.77 (27.30)	-5.08 (22.10)
Median	-5.00	0.00
25th, 75th Percentile	-7.50, 12.50	-17.50, 5.00
Min, Max	-50.0, 60.0	-42.5, 30.0
Difference in change from baseline (95%CI)		-5.85 (-27.90, 16.19)
P-value ^b		0.5867
Hedges'g (95% CI) ^c		-0.22 (-1.05, 0.61)
P-value for interaction term, treatment * [Age at Baseline]		0.4987

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	24	18
Mean (SD)	51.17 (20.94)	58.19 (21.43)
Median	48.75	60.00
25th, 75th Percentile	37.50, 67.54	37.50, 75.00
Min, Max	17.5, 100.0	20.0, 90.0
Week 26		
n	27	22
Mean (SD)	44.74 (21.51)	55.96 (19.13)
Median	45.00	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.50, 60.00	45.00, 66.67
Min, Max	10.0, 95.0	20.0, 95.0
Change from baseline to Week 26 ^a		
n	22	16
Mean (SD)	-3.87 (25.46)	1.47 (17.67)
Median	-3.75	1.25
25th, 75th Percentile	-20.00, 15.00	-8.75, 8.75
Min, Max	-52.5, 40.0	-35.0, 32.5
Week 52		
n	32	27
Mean (SD)	52.04 (22.17)	57.06 (20.82)
Median	50.00	60.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 66.25	40.00, 75.00
Min, Max	10.0, 100.0	20.0, 100.0
Change from baseline to Week 52 ^a		
n	24	17
Mean (SD)	-0.85 (25.91)	4.15 (20.45)
Median	0.00	4.17
25th, 75th Percentile	-16.25, 13.75	-7.50, 17.50
Min, Max	-50.0, 60.0	-30.0, 42.5
Difference in change from baseline (95%CI)		5.00 (-10.28, 20.27)
P-value ^b		0.5120
Hedges'g (95% CI) ^c		0.21 (-0.42, 0.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	12	12
Mean (SD)	41.39 (18.69)	39.58 (23.18)
Median	43.34	35.00
25th, 75th Percentile	30.00, 57.50	23.75, 56.25
Min, Max	2.5, 65.0	5.0, 92.5
Week 26		
n	12	11
Mean (SD)	46.67 (21.11)	39.09 (23.03)
Median	48.75	30.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.005.003_qs_sum_ovr_qol_self_cop_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 60.00	25.00, 62.50
Min, Max	0.0, 75.0	15.0, 87.5
Change from baseline to Week 26 ^a		
n	11	11
Mean (SD)	5.53 (26.98)	-1.14 (17.66)
Median	7.50	0.00
25th, 75th Percentile	0.00, 25.00	-12.50, 10.00
Min, Max	-65.0, 37.5	-32.5, 32.5
Week 52		
n	13	10
Mean (SD)	39.09 (17.38)	28.00 (14.80)
Median	37.50	26.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 47.50	20.00, 40.00
Min, Max	15.0, 72.5	7.5, 50.0
Change from baseline to Week 52 ^a		
n	12	10
Mean (SD)	-5.09 (18.62)	-12.25 (20.22)
Median	-6.25	-16.25
25th, 75th Percentile	-10.00, 7.92	-22.50, -2.50
Min, Max	-50.0, 22.5	-42.5, 22.5
Difference in change from baseline (95%CI)		-7.16 (-24.45, 10.13)
P-value ^b		0.3977
Hedges'g (95% CI) ^c		-0.36 (-1.20, 0.49)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.3125

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	4	9
Mean (SD)	52.15 (9.77)	50.00 (19.57)
Median	52.50	57.50
25th, 75th Percentile	43.75, 60.56	35.00, 57.50
Min, Max	42.5, 61.1	22.5, 85.0
Week 26		
n	6	8
Mean (SD)	46.34 (14.28)	46.15 (18.33)
Median	51.25	48.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 55.56	26.25, 63.34
Min, Max	27.5, 62.5	25.0, 67.5
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	-2.64 (9.84)	2.36 (19.96)
Median	-1.53	2.50
25th, 75th Percentile	-10.28, 5.00	2.50, 10.00
Min, Max	-15.0, 7.5	-32.5, 29.2
Week 52		
n	6	10
Mean (SD)	58.33 (19.41)	56.81 (25.86)
Median	55.00	61.95

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.00, 70.00	40.00, 75.00
Min, Max	40.0, 90.0	7.5, 100.0
Change from baseline to Week 52 ^a		
n	4	7
Mean (SD)	0.35 (6.05)	10.08 (21.93)
Median	-1.25	10.00
25th, 75th Percentile	-3.75, 4.45	-15.00, 28.89
Min, Max	-5.0, 8.9	-17.5, 42.5
Difference in change from baseline (95%CI)		9.73 (-16.13, 35.59)
P-value ^b		0.4167
Hedges'g (95% CI) ^c		0.49 (-0.77, 1.72)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	15	9
Mean (SD)	44.46 (20.74)	54.72 (28.60)
Median	42.50	60.00
25th, 75th Percentile	22.50, 65.00	35.00, 75.00
Min, Max	17.5, 75.0	5.0, 87.5
Week 26		
n	14	10
Mean (SD)	37.68 (24.25)	55.44 (21.97)
Median	31.25	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	22.50, 57.50	46.88, 67.50
Min, Max	0.0, 80.0	15.0, 85.0
Change from baseline to Week 26 ^a		
n	13	9
Mean (SD)	-4.57 (33.40)	0.49 (13.76)
Median	0.00	-2.50
25th, 75th Percentile	-20.00, 15.00	-7.50, 10.00
Min, Max	-65.0, 40.0	-20.0, 26.9
Week 52		
n	17	12
Mean (SD)	48.69 (23.26)	47.08 (19.59)
Median	47.50	42.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 62.50	30.00, 58.75
Min, Max	10.0, 87.5	25.0, 85.0
Change from baseline to Week 52 ^a		
n	15	9
Mean (SD)	1.56 (29.04)	-5.83 (22.60)
Median	5.28	2.50
25th, 75th Percentile	-24.44, 15.00	-25.00, 5.00
Min, Max	-50.0, 60.0	-32.5, 25.0
Difference in change from baseline (95%CI)		-7.39 (-30.89, 16.11)
P-value ^b		0.5212
Hedges'g (95% CI) ^c		-0.27 (-1.09, 0.57)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	11	8
Mean (SD)	55.39 (19.92)	45.94 (21.63)
Median	50.00	38.75
25th, 75th Percentile	41.67, 65.63	30.00, 57.50
Min, Max	30.0, 100.0	25.0, 90.0
Week 26		
n	13	11
Mean (SD)	51.35 (21.93)	44.32 (19.04)
Median	45.00	45.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 65.00	27.50, 62.50
Min, Max	20.0, 95.0	20.0, 72.5
Change from baseline to Week 26 ^a		
n	10	8
Mean (SD)	-0.92 (22.62)	-5.63 (20.26)
Median	2.50	-7.50
25th, 75th Percentile	-12.50, 15.00	-17.50, 3.75
Min, Max	-40.0, 30.6	-35.0, 32.5
Week 52		
n	16	11
Mean (SD)	46.29 (21.87)	47.05 (20.03)
Median	47.50	47.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	33.75, 55.00	30.00, 60.00
Min, Max	15.0, 100.0	20.0, 85.0
Change from baseline to Week 52 ^a		
n	11	7
Mean (SD)	-11.01 (23.39)	-3.93 (12.24)
Median	-7.50	-5.00
25th, 75th Percentile	-21.87, 5.83	-10.00, 2.50
Min, Max	-50.0, 22.5	-22.5, 17.5
Difference in change from baseline (95%CI)		7.08 (-13.37, 27.53)
P-value ^b		0.4735
Hedges'g (95% CI) ^c		0.34 (-0.62, 1.29)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	6	4
Mean (SD)	40.00 (25.20)	53.13 (31.52)
Median	40.00	50.00
25th, 75th Percentile	27.50, 52.50	28.75, 77.50
Min, Max	2.5, 77.5	20.0, 92.5
Week 26		
n	6	4
Mean (SD)	49.17 (15.14)	62.50 (33.48)
Median	45.00	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.00, 57.50	33.75, 91.25
Min, Max	32.5, 75.0	30.0, 95.0
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	9.17 (22.17)	9.38 (16.63)
Median	6.25	5.00
25th, 75th Percentile	-5.00, 30.00	-2.50, 21.25
Min, Max	-20.0, 37.5	-5.0, 32.5
Week 52		
n	6	4
Mean (SD)	42.50 (19.10)	42.50 (37.80)
Median	37.50	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 40.00	13.75, 71.25
Min, Max	25.0, 80.0	7.5, 92.5
Change from baseline to Week 52 ^a		
n	6	4
Mean (SD)	2.50 (12.55)	-10.63 (30.10)
Median	1.25	-15.00
25th, 75th Percentile	-7.50, 10.00	-30.00, 8.75
Min, Max	-12.5, 22.5	-42.5, 30.0
Difference in change from baseline (95%CI)		-13.13 (-44.28, 18.03)
P-value ^b		0.3598
Hedges'g (95% CI) ^c		-0.57 (-1.84, 0.74)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.5352

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	12	10
Mean (SD)	35.76 (13.80)	69.50 (16.99)
Median	35.84	68.75
25th, 75th Percentile	23.75, 46.25	60.00, 85.00
Min, Max	17.5, 60.0	35.0, 90.0
Week 26		
n	14	11
Mean (SD)	40.54 (17.07)	57.73 (23.46)
Median	38.75	55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.50, 55.00	30.00, 82.50
Min, Max	10.0, 65.0	27.5, 95.0
Change from baseline to Week 26 ^a		
n	12	8
Mean (SD)	2.57 (9.75)	-3.44 (19.50)
Median	1.25	-1.25
25th, 75th Percentile	-4.59, 11.25	-13.75, 2.50
Min, Max	-15.0, 15.0	-35.0, 32.5
Week 52		
n	15	11
Mean (SD)	47.17 (22.58)	68.18 (16.28)
Median	40.00	67.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 62.50	57.50, 85.00
Min, Max	15.0, 90.0	40.0, 92.5
Change from baseline to Week 52 ^a		
n	12	9
Mean (SD)	2.36 (16.51)	1.67 (18.07)
Median	-1.25	5.00
25th, 75th Percentile	-8.75, 11.25	-5.00, 10.00
Min, Max	-17.5, 42.5	-25.0, 30.0
Difference in change from baseline (95%CI)		-0.69 (-16.55, 15.17)
P-value ^b		0.9280
Hedges'g (95% CI) ^c		-0.04 (-0.90, 0.83)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	14	9
Mean (SD)	45.76 (18.90)	41.39 (18.88)
Median	46.25	37.50
25th, 75th Percentile	35.00, 62.50	30.00, 57.50
Min, Max	2.5, 72.5	20.0, 75.0
Week 26		
n	12	12
Mean (SD)	46.04 (23.78)	55.71 (11.95)
Median	42.50	58.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.50, 66.25	45.94, 63.75
Min, Max	0.0, 80.0	30.0, 72.5
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	4.26 (34.24)	11.51 (15.11)
Median	10.00	10.00
25th, 75th Percentile	-20.00, 37.50	2.50, 26.88
Min, Max	-65.0, 40.0	-10.0, 32.5
Week 52		
n	15	12
Mean (SD)	44.85 (22.64)	48.59 (24.18)
Median	47.50	43.34

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 55.00	35.00, 61.95
Min, Max	10.0, 82.5	7.5, 100.0
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	-1.28 (29.13)	7.84 (22.79)
Median	0.00	4.17
25th, 75th Percentile	-7.50, 12.50	-7.50, 25.00
Min, Max	-50.0, 60.0	-30.0, 42.5
Difference in change from baseline (95%CI)		9.12 (-14.78, 33.01)
P-value ^b		0.4365
Hedges'g (95% CI) ^c		0.33 (-0.52, 1.17)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	10	11
Mean (SD)	65.50 (18.35)	41.36 (23.44)
Median	65.28	37.50
25th, 75th Percentile	52.50, 77.50	25.00, 57.50
Min, Max	37.5, 100.0	5.0, 92.5
Week 26		
n	13	10
Mean (SD)	49.85 (23.03)	35.75 (23.39)
Median	47.50	26.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 60.00	20.00, 37.50
Min, Max	20.0, 95.0	15.0, 87.5
Change from baseline to Week 26 ^a		
n	10	10
Mean (SD)	-10.19 (28.80)	-6.50 (13.55)
Median	-5.28	-5.00
25th, 75th Percentile	-40.00, 15.00	-12.50, 2.50
Min, Max	-52.5, 30.6	-32.5, 10.0
Week 52		
n	15	14
Mean (SD)	52.88 (19.98)	34.82 (16.30)
Median	50.00	31.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	40.00, 62.50	22.50, 50.00
Min, Max	15.6, 100.0	7.5, 60.0
Change from baseline to Week 52 ^a		
n	10	9
Mean (SD)	-9.19 (22.65)	-15.28 (18.43)
Median	-10.00	-17.50
25th, 75th Percentile	-24.44, 8.89	-22.50, -10.00
Min, Max	-50.0, 22.5	-42.5, 22.5
Difference in change from baseline (95%CI)		-6.09 (-26.23, 14.05)
P-value ^b		0.5318
Hedges'g (95% CI) ^c		-0.28 (-1.18, 0.63)
P-value for interaction term, treatment * [Baseline AGV]		0.5408

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	25	24
Mean (SD)	48.29 (17.45)	54.79 (24.15)
Median	47.50	57.50
25th, 75th Percentile	40.00, 61.11	36.25, 73.75
Min, Max	17.5, 77.5	5.0, 92.5
Week 26		
n	28	28
Mean (SD)	46.36 (22.98)	53.43 (21.75)
Median	48.75	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.5.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 61.25	31.25, 67.09
Min, Max	0.0, 95.0	15.0, 95.0
Change from baseline to Week 26 ^a		
n	24	22
Mean (SD)	-1.22 (24.46)	0.96 (17.58)
Median	-1.25	0.00
25th, 75th Percentile	-10.00, 15.00	-10.00, 10.00
Min, Max	-65.0, 40.0	-35.0, 32.5
Week 52		
n	31	28
Mean (SD)	51.45 (23.41)	52.65 (23.80)
Median	50.00	48.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.5.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 70.00	36.25, 71.25
Min, Max	10.0, 100.0	7.5, 100.0
Change from baseline to Week 52 ^a		
n	25	22
Mean (SD)	-0.29 (24.97)	-1.86 (22.60)
Median	0.00	2.50
25th, 75th Percentile	-12.50, 12.50	-22.50, 17.50
Min, Max	-50.0, 60.0	-42.5, 42.5
Difference in change from baseline (95%CI)		-1.56 (-15.63, 12.51)
P-value ^b		0.8238
Hedges'g (95% CI) ^c		-0.06 (-0.64, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.5.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported QoLISSY : Coping Score		
Baseline		
n	11	6
Mean (SD)	47.05 (27.15)	34.58 (12.98)
Median	42.50	33.75
25th, 75th Percentile	27.50, 72.50	25.00, 37.50
Min, Max	2.5, 100.0	20.0, 57.5
Week 26		
n	11	5
Mean (SD)	42.73 (16.14)	33.00 (11.51)
Median	42.50	30.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.5.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.50, 57.50	25.00, 45.00
Min, Max	22.5, 75.0	20.0, 45.0
Change from baseline to Week 26 ^a		
n	9	5
Mean (SD)	0.56 (31.14)	-2.00 (18.15)
Median	5.00	7.50
25th, 75th Percentile	-15.00, 25.00	-5.00, 10.00
Min, Max	-52.5, 37.5	-32.5, 10.0
Week 52		
n	14	9
Mean (SD)	41.32 (15.15)	38.49 (18.52)
Median	41.25	37.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

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Table 14.2.8.2.5.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Coping Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 50.00	30.00, 55.00
Min, Max	15.6, 77.8	7.5, 63.9
Change from baseline to Week 52 ^a		
n	11	5
Mean (SD)	-6.74 (20.28)	-2.22 (18.27)
Median	-5.00	-7.50
25th, 75th Percentile	-21.87, 10.00	-12.50, -2.50
Min, Max	-50.0, 22.5	-17.5, 28.9
Difference in change from baseline (95%CI)		4.51 (-18.31, 27.33)
P-value ^b		0.6779
Hedges'g (95% CI) ^c		0.22 (-0.85, 1.27)
P-value for interaction term, treatment * [Ethnicity]		0.6679

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	19	16
Mean (SD)	70.39 (25.76)	57.42 (34.25)
Median	75.00	59.38
25th, 75th Percentile	43.75, 100.00	28.13, 90.63
Min, Max	12.5, 100.0	0.0, 100.0
Week 26		
n	20	15
Mean (SD)	70.00 (28.86)	59.17 (31.77)
Median	71.88	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.006.001_qs_sum_ovr_qol_self_bel_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.13, 96.88	25.00, 81.25
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	17	14
Mean (SD)	-3.31 (36.45)	-1.34 (21.11)
Median	0.00	0.00
25th, 75th Percentile	0.00, 6.25	-18.75, 18.75
Min, Max	-100.0, 56.3	-37.5, 25.0
Week 52		
n	25	19
Mean (SD)	62.50 (28.01)	49.01 (33.99)
Median	68.75	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 81.25	18.75, 81.25
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	19	15
Mean (SD)	-7.24 (30.50)	-4.17 (25.73)
Median	0.00	0.00
25th, 75th Percentile	-25.00, 12.50	-31.25, 18.75
Min, Max	-93.8, 37.5	-43.8, 37.5
Difference in change from baseline (95%CI)		3.07 (-16.99, 23.13)
P-value ^b		0.7572
Hedges'g (95% CI) ^c		0.11 (-0.57, 0.78)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	14	14
Mean (SD)	51.34 (25.02)	59.38 (20.03)
Median	50.00	53.13
25th, 75th Percentile	25.00, 75.00	43.75, 75.00
Min, Max	18.8, 93.8	31.3, 100.0
Week 26		
n	18	17
Mean (SD)	60.42 (30.24)	76.47 (15.07)
Median	59.38	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 87.50	68.75, 87.50
Min, Max	12.5, 100.0	50.0, 100.0
Change from baseline to Week 26 ^a		
n	12	12
Mean (SD)	12.50 (19.03)	13.54 (21.46)
Median	9.38	12.50
25th, 75th Percentile	-3.13, 25.00	6.25, 28.13
Min, Max	-12.5, 50.0	-25.0, 50.0
Week 52		
n	20	18
Mean (SD)	55.63 (27.42)	75.00 (22.28)
Median	56.25	84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.1
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Sex: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 78.13	56.25, 93.75
Min, Max	0.0, 93.8	18.8, 100.0
Change from baseline to Week 52 ^a		
n	14	12
Mean (SD)	3.57 (16.02)	18.23 (23.30)
Median	6.25	15.63
25th, 75th Percentile	0.00, 12.50	0.00, 43.75
Min, Max	-31.3, 25.0	-25.0, 43.8
Difference in change from baseline (95%CI)		14.66 (-1.33, 30.65)
P-value ^b		0.0707
Hedges'g (95% CI) ^c		0.72 (-0.08, 1.51)
P-value for interaction term, treatment *[Sex]		0.3819

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	0	2
Mean (SD)		40.63 (13.26)
Median		40.63
25th, 75th Percentile		31.25, 50.00
Min, Max		31.3, 50.0
Week 26		
n	5	7
Mean (SD)	72.50 (27.46)	73.21 (22.16)
Median	81.25	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 93.75	50.00, 93.75
Min, Max	37.5, 100.0	50.0, 100.0
Change from baseline to Week 26 ^a		
n	0	1
Mean (SD)		18.75 (NA)
Median		18.75
25th, 75th Percentile		18.75, 18.75
Min, Max		18.8, 18.8
Week 52		
n	8	11
Mean (SD)	59.38 (29.32)	53.98 (30.78)
Median	65.63	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.88, 78.13	18.75, 87.50
Min, Max	0.0, 93.8	6.3, 93.8
Change from baseline to Week 52 ^a		
n	0	2
Mean (SD)		25.00 (8.84)
Median		25.00
25th, 75th Percentile		18.75, 31.25
Min, Max		18.8, 31.3
Difference in change from baseline (95%CI)		NE
P-value ^b		NE
Hedges'g (95% CI) ^c		NE

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	21	16
Mean (SD)	60.42 (26.47)	57.81 (29.36)
Median	62.50	56.25
25th, 75th Percentile	37.50, 81.25	40.63, 81.25
Min, Max	18.8, 100.0	0.0, 100.0
Week 26		
n	22	14
Mean (SD)	60.80 (33.86)	65.18 (26.37)
Median	68.75	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 93.75	56.25, 81.25
Min, Max	0.0, 100.0	18.8, 100.0
Change from baseline to Week 26 ^a		
n	19	14
Mean (SD)	2.96 (30.50)	2.68 (16.21)
Median	6.25	6.25
25th, 75th Percentile	0.00, 12.50	-18.75, 12.50
Min, Max	-100.0, 56.3	-18.8, 31.3
Week 52		
n	24	16
Mean (SD)	58.33 (31.85)	64.45 (34.74)
Median	59.38	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 84.38	43.75, 90.63
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	21	15
Mean (SD)	-2.38 (27.28)	5.42 (24.99)
Median	0.00	6.25
25th, 75th Percentile	-12.50, 12.50	-6.25, 18.75
Min, Max	-93.8, 37.5	-43.8, 43.8
Difference in change from baseline (95%CI)		7.80 (-10.31, 25.91)
P-value ^b		0.3877
Hedges'g (95% CI) ^c		0.29 (-0.38, 0.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	12	12
Mean (SD)	65.63 (28.27)	61.98 (28.51)
Median	65.63	59.38
25th, 75th Percentile	46.88, 90.63	40.63, 87.50
Min, Max	12.5, 100.0	18.8, 100.0
Week 26		
n	11	11
Mean (SD)	71.59 (20.03)	69.32 (28.01)
Median	68.75	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 87.50	68.75, 81.25
Min, Max	43.8, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	10	11
Mean (SD)	3.75 (33.75)	7.95 (29.19)
Median	0.00	18.75
25th, 75th Percentile	-6.25, 18.75	-25.00, 25.00
Min, Max	-56.3, 56.3	-37.5, 50.0
Week 52		
n	13	10
Mean (SD)	61.54 (18.72)	65.63 (27.83)
Median	62.50	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.2
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Age at Baseline: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 75.00	50.00, 87.50
Min, Max	18.8, 87.5	6.3, 100.0
Change from baseline to Week 52 ^a		
n	12	10
Mean (SD)	-3.13 (23.61)	2.50 (31.49)
Median	0.00	0.00
25th, 75th Percentile	-28.13, 9.38	-31.25, 25.00
Min, Max	-37.5, 37.5	-43.8, 43.8
Difference in change from baseline (95%CI)		5.63 (-18.88, 30.13)
P-value ^b		0.6373
Hedges'g (95% CI) ^c		0.20 (-0.65, 1.04)
P-value for interaction term, treatment * [Age at Baseline]		0.8813

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	22	18
Mean (SD)	61.65 (23.57)	55.21 (29.41)
Median	59.38	50.00
25th, 75th Percentile	43.75, 81.25	37.50, 75.00
Min, Max	18.8, 100.0	0.0, 100.0
Week 26		
n	27	21
Mean (SD)	68.52 (28.30)	63.69 (28.27)
Median	75.00	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 93.75	50.00, 81.25
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	20	15
Mean (SD)	5.94 (31.45)	0.00 (22.53)
Median	6.25	6.25
25th, 75th Percentile	0.00, 15.63	-18.75, 18.75
Min, Max	-100.0, 56.3	-37.5, 31.3
Week 52		
n	32	27
Mean (SD)	60.16 (28.74)	54.40 (32.61)
Median	62.50	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.006.003_qs_sum_ovr_qol_self_bel_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 81.25	18.75, 87.50
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	22	17
Mean (SD)	-2.56 (26.91)	0.37 (27.11)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 12.50	-25.00, 18.75
Min, Max	-93.8, 37.5	-43.8, 43.8
Difference in change from baseline (95%CI)		2.92 (-14.74, 20.59)
P-value ^b		0.7392
Hedges'g (95% CI) ^c		0.11 (-0.53, 0.74)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	11	12
Mean (SD)	63.64 (33.64)	63.02 (26.44)
Median	75.00	65.63
25th, 75th Percentile	25.00, 100.00	43.75, 81.25
Min, Max	12.5, 100.0	18.8, 100.0
Week 26		
n	11	11
Mean (SD)	57.95 (32.49)	77.27 (16.83)
Median	68.75	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 87.50	68.75, 87.50
Min, Max	12.5, 100.0	37.5, 100.0
Change from baseline to Week 26 ^a		
n	9	11
Mean (SD)	-2.78 (31.11)	13.07 (20.24)
Median	0.00	12.50
25th, 75th Percentile	-12.50, 12.50	6.25, 25.00
Min, Max	-56.3, 56.3	-25.0, 50.0
Week 52		
n	13	10
Mean (SD)	57.69 (25.79)	81.25 (16.93)
Median	62.50	84.38

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.3
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Tanner Stage: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 75.00	75.00, 93.75
Min, Max	0.0, 87.5	43.8, 100.0
Change from baseline to Week 52 ^a		
n	11	10
Mean (SD)	-2.84 (24.10)	15.00 (24.69)
Median	0.00	12.50
25th, 75th Percentile	-25.00, 12.50	0.00, 43.75
Min, Max	-37.5, 37.5	-31.3, 43.8
Difference in change from baseline (95%CI)		17.84 (-4.46, 40.14)
P-value ^b		0.1104
Hedges'g (95% CI) ^c		0.70 (-0.19, 1.58)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.2980

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	4	9
Mean (SD)	56.25 (29.32)	56.94 (16.96)
Median	59.38	50.00
25th, 75th Percentile	31.25, 81.25	50.00, 75.00
Min, Max	25.0, 81.3	31.3, 75.0
Week 26		
n	6	8
Mean (SD)	73.96 (22.16)	78.13 (14.94)
Median	78.13	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	75.00, 87.50	68.75, 90.63
Min, Max	31.3, 93.8	56.3, 100.0
Change from baseline to Week 26 ^a		
n	4	6
Mean (SD)	10.94 (18.66)	19.79 (22.85)
Median	6.25	21.88
25th, 75th Percentile	0.00, 21.88	12.50, 31.25
Min, Max	-6.3, 37.5	-18.8, 50.0
Week 52		
n	6	10
Mean (SD)	66.67 (27.29)	75.63 (18.03)
Median	71.88	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 87.50	56.25, 87.50
Min, Max	25.0, 93.8	50.0, 100.0
Change from baseline to Week 52 ^a		
n	4	7
Mean (SD)	9.38 (8.07)	21.43 (23.62)
Median	9.38	25.00
25th, 75th Percentile	3.13, 15.63	12.50, 43.75
Min, Max	0.0, 18.8	-25.0, 43.8
Difference in change from baseline (95%CI)		12.05 (-16.08, 40.19)
P-value ^b		0.3578
Hedges'g (95% CI) ^c		0.56 (-0.71, 1.80)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	14	9
Mean (SD)	66.96 (29.26)	53.47 (31.11)
Median	75.00	43.75
25th, 75th Percentile	43.75, 93.75	37.50, 87.50
Min, Max	12.5, 100.0	0.0, 93.8
Week 26		
n	14	9
Mean (SD)	75.45 (28.43)	61.11 (33.19)
Median	84.38	62.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 100.00	50.00, 81.25
Min, Max	12.5, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	12	8
Mean (SD)	11.98 (31.81)	1.56 (24.49)
Median	9.38	9.38
25th, 75th Percentile	0.00, 34.38	-18.75, 15.63
Min, Max	-56.3, 56.3	-37.5, 37.5
Week 52		
n	17	12
Mean (SD)	61.76 (28.29)	46.35 (36.97)
Median	68.75	43.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 81.25	12.50, 87.50
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	1.34 (24.17)	3.47 (28.83)
Median	0.00	0.00
25th, 75th Percentile	-12.50, 25.00	-6.25, 18.75
Min, Max	-37.5, 37.5	-43.8, 43.8
Difference in change from baseline (95%CI)		2.13 (-21.01, 25.27)
P-value ^b		0.8498
Hedges'g (95% CI) ^c		0.08 (-0.76, 0.92)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	9	8
Mean (SD)	50.69 (23.89)	59.38 (38.67)
Median	56.25	59.38
25th, 75th Percentile	37.50, 56.25	21.88, 100.00
Min, Max	18.8, 100.0	12.5, 100.0
Week 26		
n	12	11
Mean (SD)	48.44 (29.57)	63.64 (27.22)
Median	50.00	68.75

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.25, 62.50	37.50, 81.25
Min, Max	0.0, 100.0	18.8, 100.0
Change from baseline to Week 26 ^a		
n	7	8
Mean (SD)	-8.93 (41.90)	-2.34 (21.11)
Median	0.00	0.00
25th, 75th Percentile	-6.25, 6.25	-21.88, 15.63
Min, Max	-100.0, 31.3	-31.3, 25.0
Week 52		
n	16	11
Mean (SD)	53.13 (27.58)	61.36 (33.05)
Median	59.38	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.38, 68.75	43.75, 93.75
Min, Max	0.0, 93.8	0.0, 100.0
Change from baseline to Week 52 ^a		
n	9	7
Mean (SD)	-9.03 (36.32)	-5.36 (22.66)
Median	6.25	0.00
25th, 75th Percentile	-25.00, 12.50	-25.00, 6.25
Min, Max	-93.8, 25.0	-43.8, 25.0
Difference in change from baseline (95%CI)		3.67 (-30.06, 37.40)
P-value ^b		0.8188
Hedges'g (95% CI) ^c		0.11 (-0.88, 1.10)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	6	4
Mean (SD)	72.92 (21.89)	70.31 (22.46)
Median	75.00	65.63
25th, 75th Percentile	62.50, 87.50	53.13, 87.50
Min, Max	37.5, 100.0	50.0, 100.0
Week 26		
n	6	4
Mean (SD)	67.71 (29.43)	78.13 (13.01)
Median	71.88	78.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	68.75, 81.25	68.75, 87.50
Min, Max	12.5, 100.0	62.5, 93.8
Change from baseline to Week 26 ^a		
n	6	4
Mean (SD)	-5.21 (18.29)	7.81 (12.88)
Median	3.13	6.25
25th, 75th Percentile	-25.00, 6.25	0.00, 15.63
Min, Max	-31.3, 12.5	-6.3, 25.0
Week 52		
n	6	4
Mean (SD)	62.50 (30.36)	73.44 (20.65)
Median	75.00	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.006.005_qs_sum_ovr_qol_self_bel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.8.2.6.5
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline Height Z-score: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 81.25	59.38, 87.50
Min, Max	6.3, 87.5	50.0, 100.0
Change from baseline to Week 52 ^a		
n	6	4
Mean (SD)	-10.42 (15.14)	3.13 (30.83)
Median	-6.25	0.00
25th, 75th Percentile	-25.00, 0.00	-15.63, 21.88
Min, Max	-31.3, 6.3	-31.3, 43.8
Difference in change from baseline (95%CI)		13.54 (-19.73, 46.82)
P-value ^b		0.3755
Hedges'g (95% CI) ^c		0.55 (-0.76, 1.82)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.9210

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	10	10
Mean (SD)	78.75 (20.45)	68.13 (25.25)
Median	81.25	62.50
25th, 75th Percentile	68.75, 100.00	50.00, 93.75
Min, Max	37.5, 100.0	37.5, 100.0
Week 26		
n	13	11
Mean (SD)	68.27 (33.12)	72.16 (27.30)
Median	75.00	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 93.75	68.75, 93.75
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	9	8
Mean (SD)	-9.72 (42.06)	-3.91 (26.08)
Median	6.25	-6.25
25th, 75th Percentile	-6.25, 12.50	-25.00, 18.75
Min, Max	-100.0, 37.5	-37.5, 31.3
Week 52		
n	15	11
Mean (SD)	65.00 (31.14)	69.32 (27.45)
Median	75.00	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	50.00, 87.50	56.25, 87.50
Min, Max	0.0, 100.0	6.3, 100.0
Change from baseline to Week 52 ^a		
n	10	9
Mean (SD)	-7.50 (34.96)	-3.47 (22.77)
Median	3.13	0.00
25th, 75th Percentile	-12.50, 12.50	-6.25, 0.00
Min, Max	-93.8, 25.0	-43.8, 31.3
Difference in change from baseline (95%CI)		4.03 (-24.91, 32.97)
P-value ^b		0.7726
Hedges'g (95% CI) ^c		0.13 (-0.77, 1.03)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	14	9
Mean (SD)	60.71 (27.46)	49.31 (31.78)
Median	56.25	50.00
25th, 75th Percentile	43.75, 81.25	31.25, 75.00
Min, Max	12.5, 100.0	0.0, 100.0
Week 26		
n	12	11
Mean (SD)	70.83 (25.33)	60.23 (23.93)
Median	68.75	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	59.38, 90.63	50.00, 75.00
Min, Max	12.5, 100.0	25.0, 100.0
Change from baseline to Week 26 ^a		
n	11	8
Mean (SD)	9.66 (25.21)	2.34 (20.03)
Median	6.25	9.38
25th, 75th Percentile	0.00, 18.75	-18.75, 18.75
Min, Max	-31.3, 56.3	-25.0, 25.0
Week 52		
n	15	12
Mean (SD)	60.42 (23.23)	58.85 (35.60)
Median	62.50	53.13

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 81.25	28.13, 93.75
Min, Max	18.8, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	14	9
Mean (SD)	0.89 (22.18)	11.11 (31.06)
Median	0.00	18.75
25th, 75th Percentile	-25.00, 18.75	-6.25, 37.50
Min, Max	-31.3, 37.5	-43.8, 43.8
Difference in change from baseline (95%CI)		10.22 (-12.81, 33.25)
P-value ^b		0.3667
Hedges'g (95% CI) ^c		0.38 (-0.47, 1.22)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	9	11
Mean (SD)	46.53 (23.62)	56.82 (27.02)
Median	43.75	50.00
25th, 75th Percentile	25.00, 56.25	31.25, 75.00
Min, Max	18.8, 93.8	18.8, 100.0
Week 26		
n	13	10
Mean (SD)	57.69 (29.99)	73.13 (25.52)
Median	50.00	81.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	37.50, 81.25	68.75, 87.50
Min, Max	12.5, 100.0	18.8, 100.0
Change from baseline to Week 26 ^a		
n	9	10
Mean (SD)	8.33 (23.18)	15.63 (17.98)
Median	6.25	12.50
25th, 75th Percentile	0.00, 6.25	6.25, 25.00
Min, Max	-25.0, 56.3	-6.3, 50.0
Week 52		
n	15	14
Mean (SD)	52.92 (28.53)	58.04 (31.72)
Median	56.25	71.88

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.6
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Baseline AGV Category: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	25.00, 68.75	43.75, 81.25
Min, Max	0.0, 93.8	0.0, 100.0
Change from baseline to Week 52 ^a		
n	9	9
Mean (SD)	-2.78 (20.04)	9.72 (26.35)
Median	0.00	12.50
25th, 75th Percentile	-12.50, 6.25	0.00, 25.00
Min, Max	-31.3, 37.5	-31.3, 43.8
Difference in change from baseline (95%CI)		12.50 (-10.89, 35.89)
P-value ^b		0.2740
Hedges'g (95% CI) ^c		0.51 (-0.44, 1.44)
P-value for interaction term, treatment * [Baseline AGV]		0.8801

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	23	24
Mean (SD)	64.95 (25.75)	60.94 (28.27)
Median	62.50	53.13
25th, 75th Percentile	43.75, 87.50	40.63, 87.50
Min, Max	12.5, 100.0	0.0, 100.0
Week 26		
n	27	27
Mean (SD)	66.44 (30.13)	71.06 (25.25)
Median	68.75	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

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Table 14.2.8.2.6.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 93.75	62.50, 93.75
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 26 ^a		
n	21	21
Mean (SD)	1.19 (32.99)	5.36 (23.90)
Median	6.25	6.25
25th, 75th Percentile	0.00, 12.50	-18.75, 25.00
Min, Max	-100.0, 56.3	-37.5, 50.0
Week 52		
n	31	28
Mean (SD)	59.68 (27.71)	65.40 (30.69)
Median	62.50	75.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 81.25	50.00, 87.50
Min, Max	0.0, 100.0	0.0, 100.0
Change from baseline to Week 52 ^a		
n	23	22
Mean (SD)	-6.25 (27.50)	4.26 (27.37)
Median	0.00	0.00
25th, 75th Percentile	-25.00, 6.25	-6.25, 25.00
Min, Max	-93.8, 37.5	-43.8, 43.8
Difference in change from baseline (95%CI)		10.51 (-5.99, 27.01)
P-value ^b		0.2058
Hedges'g (95% CI) ^c		0.38 (-0.22, 0.96)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
Self-Reported QoLISSY : Beliefs Score		
Baseline		
n	10	6
Mean (SD)	56.25 (29.61)	47.92 (27.00)
Median	56.25	53.13
25th, 75th Percentile	25.00, 81.25	18.75, 75.00
Min, Max	18.8, 100.0	12.5, 75.0
Week 26		
n	11	5
Mean (SD)	63.07 (29.24)	53.75 (24.04)
Median	68.75	56.25

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

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Table 14.2.8.2.6.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 87.50	37.50, 62.50
Min, Max	12.5, 100.0	25.0, 87.5
Change from baseline to Week 26 ^a		
n	8	5
Mean (SD)	8.59 (26.50)	6.25 (14.66)
Median	6.25	12.50
25th, 75th Percentile	-6.25, 21.88	6.25, 12.50
Min, Max	-31.3, 56.3	-18.8, 18.8
Week 52		
n	14	9
Mean (SD)	58.93 (28.56)	50.00 (32.48)
Median	56.25	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.006.007_qs_sum_ovr_qol_self_bel_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.8.2.6.7
Self-Reported Quality of Life in Short Statured Youth (QoLISSY) Over Time by Ethnicity: Beliefs Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.75, 81.25	18.75, 75.00
Min, Max	0.0, 100.0	6.3, 100.0
Change from baseline to Week 52 ^a		
n	10	5
Mean (SD)	5.63 (19.42)	12.50 (25.39)
Median	6.25	12.50
25th, 75th Percentile	0.00, 12.50	6.25, 25.00
Min, Max	-25.0, 37.5	-25.0, 43.8
Difference in change from baseline (95%CI)		6.88 (-18.49, 32.24)
P-value ^b		0.5682
Hedges'g (95% CI) ^c		0.30 (-0.78, 1.38)
P-value for interaction term, treatment * [Ethnicity]		0.8245

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Scores are 0-100: scores closer to 100 are indicative of a higher quality of life.

Self-reported questionnaire is not available for subjects < 8 years old.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.08.002.006.007_qs_sum_ovr_qol_self_bel_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
WeeFIM : Total Score		
Baseline		
n	33	28
Mean (SD)	112.18 (12.85)	106.82 (13.38)
Median	115.00	111.50
25th, 75th Percentile	105.00, 122.00	97.00, 117.50
Min, Max	71.0, 126.0	72.0, 123.0
Week 26		
n	31	29
Mean (SD)	113.97 (12.14)	111.07 (12.32)
Median	117.00	115.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.001_qs_sum_ovrtm_weef_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	107.00, 123.00	104.00, 120.00
Min, Max	71.0, 126.0	80.0, 126.0
Change from baseline to Week 26 ^a		
n	31	26
Mean (SD)	1.29 (6.52)	4.77 (7.89)
Median	0.00	4.50
25th, 75th Percentile	-1.00, 4.00	1.00, 9.00
Min, Max	-15.0, 19.0	-23.0, 19.0
Week 52		
n	32	30
Mean (SD)	113.47 (13.89)	110.00 (11.13)
Median	118.00	112.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.001_qs_sum_ovrtm_weef_tot_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	106.00, 124.00	102.00, 119.00
Min, Max	71.0, 126.0	82.0, 126.0
Change from baseline to Week 52 ^a		
n	32	27
Mean (SD)	1.19 (7.35)	3.30 (9.27)
Median	0.00	5.00
25th, 75th Percentile	-1.00, 3.00	0.00, 9.00
Min, Max	-12.0, 21.0	-21.0, 20.0
Difference in change from baseline (95%CI)		2.11 (-2.22, 6.44)
P-value ^b		0.3339
Hedges'g (95% CI) ^c		0.25 (-0.26, 0.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
WeeFIM : Total Score		
Baseline		
n	27	29
Mean (SD)	108.59 (14.70)	112.72 (13.32)
Median	111.00	117.00
25th, 75th Percentile	101.00, 120.00	106.00, 124.00
Min, Max	72.0, 126.0	78.0, 126.0
Week 26		
n	27	28
Mean (SD)	111.85 (14.04)	113.50 (11.65)
Median	115.00	116.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 124.00	102.00, 125.00
Min, Max	78.0, 126.0	94.0, 126.0
Change from baseline to Week 26 ^a		
n	26	28
Mean (SD)	3.04 (9.12)	0.96 (6.87)
Median	3.00	0.00
25th, 75th Percentile	0.00, 7.00	-1.00, 4.00
Min, Max	-29.0, 19.0	-21.0, 18.0
Week 52		
n	28	27
Mean (SD)	111.71 (15.14)	113.41 (11.76)
Median	115.00	116.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 125.00	102.00, 125.00
Min, Max	74.0, 126.0	92.0, 126.0
Change from baseline to Week 52 ^a		
n	27	27
Mean (SD)	2.67 (12.60)	1.33 (6.54)
Median	2.00	0.00
25th, 75th Percentile	-1.00, 8.00	-2.00, 3.00
Min, Max	-33.0, 40.0	-16.0, 21.0
Difference in change from baseline (95%CI)		-1.33 (-6.86, 4.19)
P-value ^b		0.6283
Hedges'g (95% CI) ^c		-0.13 (-0.66, 0.40)
P-value for interaction term, treatment * [Sex]		0.3216

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
WeeFIM : Total Score		
Baseline		
n	24	31
Mean (SD)	103.79 (12.79)	105.87 (13.93)
Median	107.00	106.00
25th, 75th Percentile	97.00, 112.50	94.00, 118.00
Min, Max	71.0, 123.0	78.0, 126.0
Week 26		
n	24	30
Mean (SD)	105.63 (14.20)	108.03 (12.48)
Median	107.50	106.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	99.50, 116.00	99.00, 118.00
Min, Max	71.0, 123.0	80.0, 126.0
Change from baseline to Week 26 ^a		
n	24	30
Mean (SD)	1.83 (10.37)	2.67 (8.96)
Median	0.00	3.00
25th, 75th Percentile	-2.50, 9.00	0.00, 7.00
Min, Max	-29.0, 19.0	-23.0, 19.0
Week 52		
n	24	31
Mean (SD)	104.33 (15.47)	108.10 (11.73)
Median	107.50	105.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.002_qs_sum_ovrtm_weef_tot_age_301_fas.pdf+rtf

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Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	94.50, 115.50	100.00, 118.00
Min, Max	71.0, 125.0	82.0, 126.0
Change from baseline to Week 52 ^a		
n	24	31
Mean (SD)	0.54 (11.01)	2.23 (9.67)
Median	0.00	2.00
25th, 75th Percentile	-3.50, 8.50	-2.00, 9.00
Min, Max	-33.0, 21.0	-21.0, 21.0
Difference in change from baseline (95%CI)		1.68 (-3.92, 7.29)
P-value ^b		0.5491
Hedges'g (95% CI) ^c		0.16 (-0.37, 0.69)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
WeeFIM : Total Score		
Baseline		
n	24	16
Mean (SD)	113.04 (13.61)	113.31 (13.00)
Median	118.00	116.00
25th, 75th Percentile	105.50, 122.50	110.50, 121.00
Min, Max	72.0, 126.0	72.0, 126.0
Week 26		
n	22	16
Mean (SD)	116.05 (9.77)	115.44 (9.95)
Median	117.50	116.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.002_qs_sum_ovrtm_weef_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	110.00, 126.00	110.50, 123.50
Min, Max	89.0, 126.0	90.0, 126.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	3.00 (6.17)	3.00 (5.53)
Median	0.50	3.00
25th, 75th Percentile	0.00, 6.00	0.00, 5.00
Min, Max	-5.0, 19.0	-7.0, 18.0
Week 52		
n	23	16
Mean (SD)	117.48 (10.58)	114.94 (9.71)
Median	123.00	117.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	112.00, 126.00	108.50, 122.50
Min, Max	91.0, 126.0	92.0, 126.0
Change from baseline to Week 52 ^a		
n	23	15
Mean (SD)	4.26 (11.00)	2.53 (6.17)
Median	1.00	2.00
25th, 75th Percentile	0.00, 6.00	0.00, 5.00
Min, Max	-13.0, 40.0	-9.0, 20.0
Difference in change from baseline (95%CI)		-1.73 (-7.39, 3.94)
P-value ^b		0.5402
Hedges'g (95% CI) ^c		-0.18 (-0.83, 0.47)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
WeeFIM : Total Score		
Baseline		
n	12	10
Mean (SD)	119.17 (9.20)	116.50 (9.48)
Median	123.50	120.00
25th, 75th Percentile	116.50, 125.50	112.00, 124.00
Min, Max	100.0, 126.0	99.0, 126.0
Week 26		
n	12	11
Mean (SD)	122.08 (6.50)	119.18 (8.77)
Median	124.50	123.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.002_qs_sum_ovrtm_weef_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	120.50, 126.00	116.00, 126.00
Min, Max	103.0, 126.0	99.0, 126.0
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	0.82 (2.89)	2.89 (5.78)
Median	1.00	1.00
25th, 75th Percentile	-1.00, 3.00	0.00, 4.00
Min, Max	-5.0, 6.0	-5.0, 14.0
Week 52		
n	13	10
Mean (SD)	119.46 (11.00)	117.20 (10.23)
Median	124.00	121.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.002_qs_sum_ovrtm_weef_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	119.00, 126.00	113.00, 126.00
Min, Max	89.0, 126.0	99.0, 126.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-0.08 (3.87)	2.25 (2.92)
Median	0.00	2.50
25th, 75th Percentile	-1.00, 2.00	0.00, 4.00
Min, Max	-11.0, 4.0	-2.0, 7.0
Difference in change from baseline (95%CI)		2.33 (-1.05, 5.72)
P-value ^b		0.1649
Hedges'g (95% CI) ^c		0.63 (-0.29, 1.54)
P-value for interaction term, treatment * [Age at Baseline]		0.6255

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.002_qs_sum_ovrtm_weef_tot_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
WeeFIM : Total Score		
Baseline		
n	47	46
Mean (SD)	108.79 (13.44)	107.61 (13.69)
Median	111.00	111.50
25th, 75th Percentile	102.00, 119.00	99.00, 118.00
Min, Max	71.0, 126.0	72.0, 126.0
Week 26		
n	45	46
Mean (SD)	110.93 (13.32)	110.37 (12.01)
Median	114.00	112.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.003_qs_sum_ovrtm_weef_tot_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	105.00, 121.00	102.00, 120.00
Min, Max	71.0, 126.0	80.0, 126.0
Change from baseline to Week 26 ^a		
n	44	44
Mean (SD)	1.80 (8.23)	3.27 (8.16)
Median	0.00	4.00
25th, 75th Percentile	-0.50, 6.00	0.00, 6.50
Min, Max	-29.0, 19.0	-23.0, 19.0
Week 52		
n	47	47
Mean (SD)	110.74 (14.77)	109.68 (11.15)
Median	115.00	111.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.003_qs_sum_ovrtm_weef_tot_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 123.00	102.00, 119.00
Min, Max	71.0, 126.0	82.0, 126.0
Change from baseline to Week 52 ^a		
n	46	45
Mean (SD)	1.67 (10.88)	2.42 (8.68)
Median	0.00	2.00
25th, 75th Percentile	-1.00, 8.00	0.00, 6.00
Min, Max	-33.0, 40.0	-21.0, 21.0
Difference in change from baseline (95%CI)		0.75 (-3.36, 4.85)
P-value ^b		0.7180
Hedges'g (95% CI) ^c		0.08 (-0.34, 0.49)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.003_qs_sum_ovrtm_weef_tot_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
WeeFIM : Total Score		
Baseline		
n	13	11
Mean (SD)	117.00 (13.22)	119.09 (8.30)
Median	124.00	121.00
25th, 75th Percentile	118.00, 125.00	118.00, 125.00
Min, Max	91.0, 126.0	99.0, 126.0
Week 26		
n	13	11
Mean (SD)	120.08 (8.92)	120.18 (8.06)
Median	124.00	123.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.003_qs_sum_ovrtm_weef_tot_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	120.00, 126.00	116.00, 126.00
Min, Max	102.0, 126.0	99.0, 126.0
Change from baseline to Week 26 ^a		
n	13	10
Mean (SD)	3.08 (6.25)	0.70 (3.43)
Median	1.00	0.00
25th, 75th Percentile	0.00, 6.00	0.00, 2.00
Min, Max	-5.0, 19.0	-5.0, 6.0
Week 52		
n	13	10
Mean (SD)	119.54 (10.81)	120.70 (8.42)
Median	125.00	124.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.003_qs_sum_ovrtm_weef_tot_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	117.00, 126.00	120.00, 126.00
Min, Max	91.0, 126.0	99.0, 126.0
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	2.54 (6.49)	1.78 (3.15)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.00	0.00, 3.00
Min, Max	-1.0, 23.0	-3.0, 7.0
Difference in change from baseline (95%CI)		-0.76 (-5.13, 3.61)
P-value ^b		0.7193
Hedges'g (95% CI) ^c		-0.14 (-0.98, 0.72)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.7341

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
WeeFIM : Total Score		
Baseline		
n	10	14
Mean (SD)	104.90 (12.53)	113.86 (11.65)
Median	107.00	117.50
25th, 75th Percentile	102.00, 107.00	111.00, 121.00
Min, Max	76.0, 126.0	83.0, 125.0
Week 26		
n	10	13
Mean (SD)	104.40 (15.46)	112.85 (10.99)
Median	107.00	116.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	103.00, 111.00	104.00, 121.00
Min, Max	78.0, 125.0	94.0, 125.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-0.50 (11.59)	-0.50 (8.54)
Median	1.00	0.00
25th, 75th Percentile	-3.00, 6.00	-4.00, 4.50
Min, Max	-29.0, 15.0	-21.0, 14.0
Week 52		
n	10	13
Mean (SD)	105.00 (15.13)	112.08 (11.12)
Median	108.50	113.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

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Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 112.00	102.00, 121.00
Min, Max	74.0, 125.0	93.0, 125.0
Change from baseline to Week 52 ^a		
n	10	12
Mean (SD)	0.10 (12.76)	-0.75 (6.57)
Median	1.50	0.00
25th, 75th Percentile	-2.00, 9.00	-2.50, 3.00
Min, Max	-33.0, 10.0	-16.0, 10.0
Difference in change from baseline (95%CI)		-0.85 (-10.49, 8.79)
P-value ^b		0.8517
Hedges'g (95% CI) ^c		-0.08 (-0.92, 0.76)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
WeeFIM : Total Score		
Baseline		
n	23	18
Mean (SD)	112.39 (13.44)	101.50 (14.47)
Median	115.00	102.50
25th, 75th Percentile	109.00, 123.00	91.00, 114.00
Min, Max	72.0, 126.0	72.0, 123.0
Week 26		
n	22	18
Mean (SD)	115.32 (10.97)	106.67 (13.55)
Median	118.00	107.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	105.00, 125.00	96.00, 117.00
Min, Max	89.0, 126.0	80.0, 126.0
Change from baseline to Week 26 ^a		
n	21	18
Mean (SD)	1.67 (6.63)	5.17 (9.58)
Median	0.00	4.50
25th, 75th Percentile	0.00, 4.00	1.00, 11.00
Min, Max	-15.0, 17.0	-23.0, 19.0
Week 52		
n	23	18
Mean (SD)	115.17 (12.05)	106.50 (12.22)
Median	118.00	105.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	109.00, 125.00	99.00, 115.00
Min, Max	84.0, 126.0	82.0, 125.0
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	2.23 (9.99)	5.00 (9.47)
Median	0.00	4.50
25th, 75th Percentile	0.00, 2.00	1.00, 11.00
Min, Max	-11.0, 40.0	-21.0, 21.0
Difference in change from baseline (95%CI)		2.77 (-3.51, 9.05)
P-value ^b		0.3772
Hedges'g (95% CI) ^c		0.28 (-0.35, 0.90)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
WeeFIM : Total Score		
Baseline		
n	19	21
Mean (SD)	109.63 (15.39)	113.29 (11.51)
Median	117.00	116.00
25th, 75th Percentile	98.00, 122.00	111.00, 121.00
Min, Max	71.0, 126.0	87.0, 126.0
Week 26		
n	18	21
Mean (SD)	113.33 (13.68)	115.62 (9.99)
Median	116.00	118.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	110.00, 123.00	107.00, 125.00
Min, Max	71.0, 126.0	99.0, 126.0
Change from baseline to Week 26 ^a		
n	18	20
Mean (SD)	4.11 (7.68)	3.15 (4.45)
Median	0.50	2.00
25th, 75th Percentile	0.00, 11.00	0.00, 6.00
Min, Max	-6.0, 19.0	-5.0, 13.0
Week 52		
n	19	21
Mean (SD)	113.21 (15.56)	114.71 (9.71)
Median	119.00	117.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

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Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 125.00	105.00, 123.00
Min, Max	71.0, 126.0	96.0, 126.0
Change from baseline to Week 52 ^a		
n	19	20
Mean (SD)	3.58 (9.77)	2.45 (7.61)
Median	0.00	3.50
25th, 75th Percentile	-1.00, 8.00	0.00, 6.50
Min, Max	-13.0, 23.0	-17.0, 15.0
Difference in change from baseline (95%CI)		-1.13 (-6.79, 4.54)
P-value ^b		0.6887
Hedges'g (95% CI) ^c		-0.13 (-0.75, 0.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.005_qs_sum_ovrtm_weef_tot_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
WeeFIM : Total Score		
Baseline		
n	8	4
Mean (SD)	114.63 (11.29)	115.00 (12.73)
Median	119.50	115.50
25th, 75th Percentile	104.50, 123.50	104.00, 126.00
Min, Max	96.0, 126.0	103.0, 126.0
Week 26		
n	8	5
Mean (SD)	116.50 (10.62)	116.80 (11.43)
Median	120.00	123.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	112.00, 124.00	107.00, 126.00
Min, Max	94.0, 126.0	102.0, 126.0
Change from baseline to Week 26 ^a		
n	8	4
Mean (SD)	1.88 (4.82)	0.25 (2.87)
Median	0.50	0.00
25th, 75th Percentile	-1.00, 5.50	-1.50, 2.00
Min, Max	-5.0, 10.0	-3.0, 4.0
Week 52		
n	8	5
Mean (SD)	113.63 (16.29)	115.80 (13.08)
Median	122.50	124.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	102.50, 124.50	102.00, 126.00
Min, Max	84.0, 126.0	101.0, 126.0
Change from baseline to Week 52 ^a		
n	8	4
Mean (SD)	-1.00 (7.54)	-1.25 (1.50)
Median	-0.50	-1.00
25th, 75th Percentile	-6.50, 5.00	-2.50, 0.00
Min, Max	-12.0, 8.0	-3.0, 0.0
Difference in change from baseline (95%CI)		-0.25 (-6.63, 6.13)
P-value ^b		0.9303
Hedges'g (95% CI) ^c		-0.04 (-1.24, 1.16)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.7883

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
WeeFIM : Total Score		
Baseline		
n	19	18
Mean (SD)	113.63 (11.67)	111.00 (11.60)
Median	118.00	113.50
25th, 75th Percentile	107.00, 122.00	103.00, 120.00
Min, Max	91.0, 126.0	86.0, 123.0
Week 26		
n	19	17
Mean (SD)	114.79 (10.43)	110.94 (14.66)
Median	117.00	116.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 125.00	100.00, 123.00
Min, Max	94.0, 126.0	80.0, 126.0
Change from baseline to Week 26 ^a		
n	19	16
Mean (SD)	1.16 (5.01)	1.56 (10.20)
Median	0.00	3.50
25th, 75th Percentile	-3.00, 4.00	0.50, 6.50
Min, Max	-5.0, 13.0	-23.0, 14.0
Week 52		
n	19	18
Mean (SD)	116.47 (11.11)	109.28 (12.51)
Median	118.00	108.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	112.00, 126.00	102.00, 119.00
Min, Max	84.0, 126.0	82.0, 126.0
Change from baseline to Week 52 ^a		
n	19	17
Mean (SD)	2.84 (7.10)	-0.82 (9.79)
Median	1.00	2.00
25th, 75th Percentile	-1.00, 8.00	-3.00, 5.00
Min, Max	-12.0, 23.0	-21.0, 15.0
Difference in change from baseline (95%CI)		-3.67 (-9.42, 2.09)
P-value ^b		0.2039
Hedges'g (95% CI) ^c		-0.42 (-1.08, 0.24)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
WeeFIM : Total Score		
Baseline		
n	17	13
Mean (SD)	112.76 (14.21)	106.85 (17.57)
Median	115.00	111.00
25th, 75th Percentile	106.00, 125.00	94.00, 124.00
Min, Max	71.0, 126.0	72.0, 126.0
Week 26		
n	16	14
Mean (SD)	116.94 (13.93)	111.71 (12.62)
Median	121.00	112.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	115.00, 126.00	104.00, 125.00
Min, Max	71.0, 126.0	90.0, 126.0
Change from baseline to Week 26 ^a		
n	15	13
Mean (SD)	2.47 (5.03)	4.23 (6.30)
Median	0.00	4.00
25th, 75th Percentile	0.00, 4.00	0.00, 8.00
Min, Max	-5.0, 15.0	-7.0, 18.0
Week 52		
n	17	13
Mean (SD)	114.82 (15.74)	109.92 (12.62)
Median	124.00	111.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 124.00	100.00, 121.00
Min, Max	71.0, 126.0	92.0, 126.0
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	1.25 (5.81)	4.33 (6.20)
Median	0.00	3.00
25th, 75th Percentile	-0.50, 3.00	0.00, 6.00
Min, Max	-11.0, 18.0	-3.0, 20.0
Difference in change from baseline (95%CI)		3.08 (-1.61, 7.78)
P-value ^b		0.1886
Hedges'g (95% CI) ^c		0.50 (-0.26, 1.26)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
WeeFIM : Total Score		
Baseline		
n	24	26
Mean (SD)	106.58 (14.39)	110.50 (12.88)
Median	109.00	113.50
25th, 75th Percentile	99.50, 118.00	105.00, 119.00
Min, Max	72.0, 126.0	78.0, 126.0
Week 26		
n	23	26
Mean (SD)	108.74 (13.50)	113.42 (9.84)
Median	110.00	115.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	101.00, 120.00	103.00, 121.00
Min, Max	78.0, 126.0	96.0, 126.0
Change from baseline to Week 26 ^a		
n	23	25
Mean (SD)	2.61 (10.80)	2.84 (6.24)
Median	1.00	1.00
25th, 75th Percentile	0.00, 8.00	0.00, 5.00
Min, Max	-29.0, 19.0	-5.0, 19.0
Week 52		
n	24	26
Mean (SD)	108.08 (15.00)	114.08 (9.98)
Median	112.00	115.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.006_qs_sum_ovrtm_weef_tot_bhagv_301_fas.pdf+rtf

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Table 14.2.9.1.1.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	98.00, 120.50	104.00, 123.00
Min, Max	74.0, 126.0	93.0, 126.0
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	1.50 (13.82)	3.48 (7.04)
Median	0.00	2.00
25th, 75th Percentile	-6.50, 8.00	0.00, 7.00
Min, Max	-33.0, 40.0	-15.0, 21.0
Difference in change from baseline (95%CI)		1.98 (-4.43, 8.39)
P-value ^b		0.5342
Hedges'g (95% CI) ^c		0.18 (-0.38, 0.74)
P-value for interaction term, treatment * [Baseline AGV]		0.2602

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
WeeFIM : Total Score		
Baseline		
n	40	42
Mean (SD)	109.78 (13.62)	110.26 (13.19)
Median	113.50	114.00
25th, 75th Percentile	102.00, 120.00	102.00, 121.00
Min, Max	72.0, 126.0	72.0, 126.0
Week 26		
n	39	43
Mean (SD)	112.21 (12.99)	112.98 (11.66)
Median	115.00	116.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 125.00	102.00, 123.00
Min, Max	78.0, 126.0	87.0, 126.0
Change from baseline to Week 26 ^a		
n	38	40
Mean (SD)	2.00 (7.82)	3.03 (6.20)
Median	1.00	3.00
25th, 75th Percentile	0.00, 6.00	0.00, 5.50
Min, Max	-29.0, 17.0	-21.0, 18.0
Week 52		
n	41	43
Mean (SD)	111.41 (14.78)	111.74 (11.03)
Median	114.00	114.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	104.00, 124.00	102.00, 123.00
Min, Max	74.0, 126.0	92.0, 126.0
Change from baseline to Week 52 ^a		
n	40	40
Mean (SD)	1.33 (11.39)	1.98 (7.47)
Median	0.50	2.00
25th, 75th Percentile	-1.00, 6.00	-1.00, 6.00
Min, Max	-33.0, 40.0	-17.0, 20.0
Difference in change from baseline (95%CI)		0.65 (-3.65, 4.95)
P-value ^b		0.7637
Hedges'g (95% CI) ^c		0.07 (-0.37, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Table 14.2.9.1.1.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
WeeFIM : Total Score		
Baseline		
n	20	15
Mean (SD)	112.15 (14.09)	108.60 (14.96)
Median	113.50	111.00
25th, 75th Percentile	105.50, 123.50	100.00, 119.00
Min, Max	71.0, 126.0	78.0, 126.0
Week 26		
n	19	14
Mean (SD)	114.58 (13.16)	110.07 (13.01)
Median	118.00	112.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

Report: mi897809 27JUL2023 07:13 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.001.007_qs_sum_ovrtm_weef_tot_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	110.00, 124.00	104.00, 120.00
Min, Max	71.0, 126.0	80.0, 126.0
Change from baseline to Week 26 ^a		
n	19	14
Mean (SD)	2.26 (7.93)	2.14 (10.81)
Median	0.00	0.50
25th, 75th Percentile	-1.00, 7.00	-3.00, 7.00
Min, Max	-15.0, 19.0	-23.0, 19.0
Week 52		
n	19	14
Mean (SD)	115.32 (13.50)	111.21 (13.12)
Median	118.00	114.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.1.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Total Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	112.00, 124.00	105.00, 121.00
Min, Max	71.0, 126.0	82.0, 126.0
Change from baseline to Week 52 ^a		
n	19	14
Mean (SD)	3.00 (6.42)	3.29 (9.61)
Median	0.00	2.50
25th, 75th Percentile	-1.00, 8.00	0.00, 7.00
Min, Max	-4.0, 21.0	-21.0, 21.0
Difference in change from baseline (95%CI)		0.29 (-5.40, 5.97)
P-value ^b		0.9190
Hedges'g (95% CI) ^c		0.04 (-0.66, 0.73)
P-value for interaction term, treatment * [Ethnicity]		0.9244

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-126: A higher score reflects a higher level of independence.

Total score is the sum of the mean physical social and emotional scores, divided by 3.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
WeeFIM : Self-Care Score		
Baseline		
n	33	28
Mean (SD)	47.64 (8.03)	43.79 (8.90)
Median	49.00	46.00
25th, 75th Percentile	40.00, 56.00	36.00, 51.50
Min, Max	30.0, 56.0	29.0, 56.0
Week 26		
n	31	29
Mean (SD)	48.48 (7.50)	45.72 (8.85)
Median	50.00	46.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.00, 56.00	40.00, 53.00
Min, Max	30.0, 56.0	21.0, 56.0
Change from baseline to Week 26 ^a		
n	31	26
Mean (SD)	0.52 (4.28)	2.35 (5.34)
Median	0.00	2.50
25th, 75th Percentile	-1.00, 1.00	0.00, 5.00
Min, Max	-10.0, 13.0	-20.0, 10.0
Week 52		
n	32	30
Mean (SD)	49.38 (7.36)	45.70 (8.47)
Median	52.50	46.00
25th, 75th Percentile	42.50, 56.00	39.00, 55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 56.0	23.0, 56.0
Change from baseline to Week 52 ^a		
n	32	27
Mean (SD)	1.72 (4.77)	2.19 (6.37)
Median	0.00	4.00
25th, 75th Percentile	0.00, 2.00	0.00, 6.00
Min, Max	-5.0, 18.0	-18.0, 10.0
Difference in change from baseline (95%CI)		0.47 (-2.44, 3.38)
P-value ^b		0.7494
Hedges'g (95% CI) ^c		0.08 (-0.43, 0.59)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
WeeFIM : Self-Care Score		
Baseline		
n	27	29
Mean (SD)	45.63 (8.74)	46.97 (9.34)
Median	46.00	50.00
25th, 75th Percentile	39.00, 53.00	42.00, 54.00
Min, Max	22.0, 56.0	24.0, 56.0
Week 26		
n	27	28
Mean (SD)	47.70 (8.59)	47.21 (8.86)
Median	49.00	49.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	43.00, 56.00	41.00, 56.00
Min, Max	23.0, 56.0	31.0, 56.0
Change from baseline to Week 26 ^a		
n	26	28
Mean (SD)	1.92 (5.11)	0.46 (5.64)
Median	0.50	0.00
25th, 75th Percentile	0.00, 6.00	-1.00, 2.00
Min, Max	-10.0, 10.0	-17.0, 15.0
Week 52		
n	28	27
Mean (SD)	48.68 (7.29)	48.04 (8.08)
Median	49.00	50.00
25th, 75th Percentile	44.00, 56.00	42.00, 56.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 56.0	33.0, 56.0
Change from baseline to Week 52 ^a		
n	27	27
Mean (SD)	2.78 (5.32)	1.59 (4.47)
Median	1.00	2.00
25th, 75th Percentile	0.00, 5.00	0.00, 4.00
Min, Max	-6.0, 18.0	-12.0, 11.0
Difference in change from baseline (95%CI)		-1.19 (-3.87, 1.50)
P-value ^b		0.3795
Hedges'g (95% CI) ^c		-0.24 (-0.77, 0.30)
P-value for interaction term, treatment * [Sex]		0.4073

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
WeeFIM : Self-Care Score		
Baseline		
n	24	31
Mean (SD)	41.75 (7.76)	42.26 (9.34)
Median	40.50	42.00
25th, 75th Percentile	39.00, 44.50	34.00, 51.00
Min, Max	22.0, 56.0	24.0, 56.0
Week 26		
n	24	30
Mean (SD)	43.33 (8.67)	43.17 (9.48)
Median	46.50	43.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	36.50, 49.50	35.00, 51.00
Min, Max	23.0, 56.0	21.0, 56.0
Change from baseline to Week 26 ^a		
n	24	30
Mean (SD)	1.58 (6.11)	1.23 (6.96)
Median	0.00	2.00
25th, 75th Percentile	-1.00, 8.00	0.00, 5.00
Min, Max	-10.0, 13.0	-20.0, 15.0
Week 52		
n	24	31
Mean (SD)	44.21 (7.34)	43.81 (8.30)
Median	45.00	42.00
25th, 75th Percentile	39.50, 49.50	38.00, 50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 56.0	23.0, 56.0
Change from baseline to Week 52 ^a		
n	24	31
Mean (SD)	2.46 (4.53)	1.55 (6.69)
Median	1.50	3.00
25th, 75th Percentile	0.00, 5.50	0.00, 6.00
Min, Max	-5.0, 15.0	-18.0, 11.0
Difference in change from baseline (95%CI)		-0.91 (-4.10, 2.28)
P-value ^b		0.5699
Hedges'g (95% CI) ^c		-0.15 (-0.69, 0.38)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
WeeFIM : Self-Care Score		
Baseline		
n	24	16
Mean (SD)	48.92 (7.58)	48.69 (7.59)
Median	51.00	50.00
25th, 75th Percentile	45.00, 56.00	46.50, 54.50
Min, Max	31.0, 56.0	31.0, 56.0
Week 26		
n	22	16
Mean (SD)	50.05 (5.93)	49.19 (6.35)
Median	51.00	49.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	44.00, 56.00	46.50, 54.50
Min, Max	39.0, 56.0	34.0, 56.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	1.09 (4.02)	1.20 (2.73)
Median	0.00	0.00
25th, 75th Percentile	0.00, 2.00	0.00, 3.00
Min, Max	-8.0, 10.0	-3.0, 8.0
Week 52		
n	23	16
Mean (SD)	51.61 (5.23)	49.63 (6.81)
Median	54.00	52.50
25th, 75th Percentile	47.00, 56.00	46.00, 55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	40.0, 56.0	35.0, 56.0
Change from baseline to Week 52 ^a		
n	23	15
Mean (SD)	2.61 (6.54)	1.93 (3.22)
Median	0.00	2.00
25th, 75th Percentile	-1.00, 4.00	0.00, 4.00
Min, Max	-6.0, 18.0	-4.0, 7.0
Difference in change from baseline (95%CI)		-0.68 (-3.92, 2.57)
P-value ^b		0.6751
Hedges'g (95% CI) ^c		-0.12 (-0.77, 0.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
WeeFIM : Self-Care Score		
Baseline		
n	12	10
Mean (SD)	52.33 (5.33)	49.90 (7.89)
Median	54.50	53.50
25th, 75th Percentile	50.50, 56.00	49.00, 55.00
Min, Max	38.0, 56.0	35.0, 56.0
Week 26		
n	12	11
Mean (SD)	54.17 (2.62)	51.45 (6.44)
Median	55.50	54.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	53.50, 56.00	50.00, 56.00
Min, Max	49.0, 56.0	35.0, 56.0
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	0.36 (1.36)	2.11 (3.66)
Median	0.00	2.00
25th, 75th Percentile	0.00, 1.00	0.00, 2.00
Min, Max	-2.0, 3.0	-3.0, 10.0
Week 52		
n	13	10
Mean (SD)	53.46 (5.13)	51.60 (7.11)
Median	56.00	55.50
25th, 75th Percentile	53.00, 56.00	47.00, 56.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.002_qs_sum_ovrtm_weef_sel_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	38.0, 56.0	35.0, 56.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	0.92 (1.31)	3.13 (3.31)
Median	0.00	2.00
25th, 75th Percentile	0.00, 2.00	0.50, 5.50
Min, Max	0.0, 4.0	0.0, 9.0
Difference in change from baseline (95%CI)		2.21 (-0.60, 5.02)
P-value ^b		0.1085
Hedges'g (95% CI) ^c		0.92 (-0.04, 1.85)
P-value for interaction term, treatment *[Age at Baseline]		0.5233

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
WeeFIM : Self-Care Score		
Baseline		
n	47	46
Mean (SD)	45.15 (8.23)	44.00 (9.26)
Median	44.00	46.50
25th, 75th Percentile	39.00, 53.00	36.00, 52.00
Min, Max	22.0, 56.0	24.0, 56.0
Week 26		
n	45	46
Mean (SD)	46.44 (8.08)	45.13 (8.86)
Median	48.00	46.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.003_qs_sum_ovrtm_weef_sel_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.00, 53.00	40.00, 53.00
Min, Max	23.0, 56.0	21.0, 56.0
Change from baseline to Week 26 ^a		
n	44	44
Mean (SD)	1.07 (5.00)	1.52 (6.02)
Median	0.00	1.50
25th, 75th Percentile	0.00, 3.00	0.00, 5.00
Min, Max	-10.0, 13.0	-20.0, 15.0
Week 52		
n	47	47
Mean (SD)	47.72 (7.42)	45.55 (8.13)
Median	49.00	46.00
25th, 75th Percentile	42.00, 55.00	39.00, 54.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.003_qs_sum_ovrtm_weef_sel_tan_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 56.0	23.0, 56.0
Change from baseline to Week 52 ^a		
n	46	45
Mean (SD)	2.43 (5.24)	1.93 (5.86)
Median	0.50	3.00
25th, 75th Percentile	0.00, 4.00	0.00, 6.00
Min, Max	-6.0, 18.0	-18.0, 11.0
Difference in change from baseline (95%CI)		-0.50 (-2.81, 1.81)
P-value ^b		0.6677
Hedges'g (95% CI) ^c		-0.09 (-0.50, 0.32)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
WeeFIM : Self-Care Score		
Baseline		
n	13	11
Mean (SD)	52.46 (6.08)	51.27 (6.26)
Median	56.00	53.00
25th, 75th Percentile	52.00, 56.00	49.00, 56.00
Min, Max	35.0, 56.0	35.0, 56.0
Week 26		
n	13	11
Mean (SD)	53.92 (3.82)	52.00 (6.23)
Median	56.00	54.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	54.00, 56.00	50.00, 56.00
Min, Max	43.0, 56.0	35.0, 56.0
Change from baseline to Week 26 ^a		
n	13	10
Mean (SD)	1.46 (3.55)	0.70 (2.50)
Median	0.00	0.50
25th, 75th Percentile	0.00, 1.00	0.00, 2.00
Min, Max	-2.0, 10.0	-3.0, 5.0
Week 52		
n	13	10
Mean (SD)	53.85 (4.14)	52.70 (6.62)
Median	56.00	56.00
25th, 75th Percentile	54.00, 56.00	53.00, 56.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.003_qs_sum_ovrtm_weef_sel_tan_301_fas.pdf+rtf

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Table 14.2.9.1.2.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	42.0, 56.0	35.0, 56.0
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	1.38 (4.19)	1.67 (3.00)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.00	0.00, 3.00
Min, Max	-4.0, 14.0	-3.0, 7.0
Difference in change from baseline (95%CI)		0.28 (-3.12, 3.69)
P-value ^b		0.8645
Hedges'g (95% CI) ^c		0.07 (-0.78, 0.92)
P-value for interaction term, treatment *[Baseline Tanner Stage]		0.7582

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
WeeFIM : Self-Care Score		
Baseline		
n	10	14
Mean (SD)	41.30 (8.81)	48.57 (7.93)
Median	41.50	49.50
25th, 75th Percentile	39.00, 44.00	47.00, 53.00
Min, Max	22.0, 56.0	28.0, 56.0
Week 26		
n	10	13
Mean (SD)	42.00 (10.19)	47.62 (7.64)
Median	43.00	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.005_qs_sum_ovrtm_weef_sel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 50.00	46.00, 53.00
Min, Max	23.0, 56.0	31.0, 56.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	0.70 (5.72)	-0.17 (7.25)
Median	0.50	0.00
25th, 75th Percentile	-3.00, 6.00	-2.50, 1.50
Min, Max	-10.0, 10.0	-17.0, 15.0
Week 52		
n	10	13
Mean (SD)	44.50 (8.03)	48.00 (7.27)
Median	47.00	52.00
25th, 75th Percentile	41.00, 48.00	45.00, 53.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.005_qs_sum_ovrtm_weef_sel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 56.0	33.0, 56.0
Change from baseline to Week 52 ^a		
n	10	12
Mean (SD)	3.20 (4.78)	0.50 (5.09)
Median	2.50	1.00
25th, 75th Percentile	0.00, 7.00	-1.50, 4.50
Min, Max	-3.0, 11.0	-12.0, 6.0
Difference in change from baseline (95%CI)		-2.70 (-7.12, 1.72)
P-value ^b		0.2175
Hedges'g (95% CI) ^c		-0.52 (-1.37, 0.34)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
WeeFIM : Self-Care Score		
Baseline		
n	23	18
Mean (SD)	48.13 (7.65)	40.56 (9.30)
Median	50.00	39.50
25th, 75th Percentile	44.00, 54.00	34.00, 48.00
Min, Max	31.0, 56.0	24.0, 56.0
Week 26		
n	22	18
Mean (SD)	49.45 (6.83)	42.00 (9.74)
Median	51.50	42.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.00, 56.00	34.00, 49.00
Min, Max	35.0, 56.0	21.0, 56.0
Change from baseline to Week 26 ^a		
n	21	18
Mean (SD)	0.48 (4.17)	1.44 (6.15)
Median	0.00	1.50
25th, 75th Percentile	0.00, 1.00	0.00, 5.00
Min, Max	-10.0, 8.0	-20.0, 10.0
Week 52		
n	23	18
Mean (SD)	50.00 (6.85)	42.67 (8.95)
Median	53.00	41.00
25th, 75th Percentile	46.00, 56.00	39.00, 50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.005_qs_sum_ovrtm_weef_sel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	38.0, 56.0	23.0, 56.0
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	1.55 (4.22)	2.11 (6.39)
Median	0.00	2.50
25th, 75th Percentile	0.00, 2.00	0.00, 5.00
Min, Max	-5.0, 18.0	-18.0, 11.0
Difference in change from baseline (95%CI)		0.57 (-2.84, 3.98)
P-value ^b		0.7388
Hedges'g (95% CI) ^c		0.10 (-0.52, 0.73)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
WeeFIM : Self-Care Score		
Baseline		
n	19	21
Mean (SD)	46.79 (8.52)	47.14 (8.56)
Median	49.00	51.00
25th, 75th Percentile	39.00, 56.00	44.00, 53.00
Min, Max	30.0, 56.0	29.0, 56.0
Week 26		
n	18	21
Mean (SD)	49.22 (7.11)	48.62 (7.98)
Median	49.00	51.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	44.00, 56.00	43.00, 56.00
Min, Max	30.0, 56.0	31.0, 56.0
Change from baseline to Week 26 ^a		
n	18	20
Mean (SD)	2.61 (5.09)	2.00 (3.87)
Median	0.00	2.00
25th, 75th Percentile	0.00, 8.00	0.00, 5.00
Min, Max	-8.0, 13.0	-11.0, 6.0
Week 52		
n	19	21
Mean (SD)	49.68 (7.17)	48.43 (7.81)
Median	51.00	50.00
25th, 75th Percentile	44.00, 56.00	40.00, 56.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 56.0	35.0, 56.0
Change from baseline to Week 52 ^a		
n	19	20
Mean (SD)	2.89 (6.44)	2.15 (5.14)
Median	0.00	4.00
25th, 75th Percentile	0.00, 4.00	0.00, 6.00
Min, Max	-6.0, 18.0	-12.0, 8.0
Difference in change from baseline (95%CI)		-0.74 (-4.52, 3.03)
P-value ^b		0.6913
Hedges'g (95% CI) ^c		-0.13 (-0.75, 0.50)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.005_qs_sum_ovrtm_weef_sel_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
WeeFIM : Self-Care Score		
Baseline		
n	8	4
Mean (SD)	49.38 (7.73)	47.00 (10.52)
Median	53.00	48.00
25th, 75th Percentile	41.50, 56.00	38.00, 56.00
Min, Max	38.0, 56.0	36.0, 56.0
Week 26		
n	8	5
Mean (SD)	49.63 (7.44)	50.40 (7.13)
Median	52.00	54.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.50, 55.00	46.00, 56.00
Min, Max	34.0, 56.0	40.0, 56.0
Change from baseline to Week 26 ^a		
n	8	4
Mean (SD)	0.25 (3.62)	2.50 (5.00)
Median	0.00	0.00
25th, 75th Percentile	-2.00, 1.00	0.00, 5.00
Min, Max	-4.0, 8.0	0.0, 10.0
Week 52		
n	8	5
Mean (SD)	50.50 (6.87)	51.80 (5.76)
Median	54.00	56.00
25th, 75th Percentile	44.50, 55.50	46.00, 56.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	40.0, 56.0	45.0, 56.0
Change from baseline to Week 52 ^a		
n	8	4
Mean (SD)	1.13 (3.68)	3.75 (4.50)
Median	0.00	3.00
25th, 75th Percentile	-1.00, 2.50	0.00, 7.50
Min, Max	-3.0, 9.0	0.0, 9.0
Difference in change from baseline (95%CI)		2.63 (-2.76, 8.01)
P-value ^b		0.3027
Hedges'g (95% CI) ^c		0.61 (-0.63, 1.83)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.5243

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
WeeFIM : Self-Care Score		
Baseline		
n	19	18
Mean (SD)	47.84 (8.52)	46.33 (7.97)
Median	52.00	48.50
25th, 75th Percentile	39.00, 56.00	41.00, 52.00
Min, Max	33.0, 56.0	31.0, 56.0
Week 26		
n	19	17
Mean (SD)	48.16 (7.73)	45.41 (10.61)
Median	52.00	48.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.00, 56.00	38.00, 55.00
Min, Max	34.0, 56.0	21.0, 56.0
Change from baseline to Week 26 ^a		
n	19	16
Mean (SD)	0.32 (3.96)	0.19 (7.90)
Median	0.00	1.50
25th, 75th Percentile	-2.00, 1.00	0.00, 5.50
Min, Max	-8.0, 8.0	-20.0, 10.0
Week 52		
n	19	18
Mean (SD)	50.42 (6.44)	46.11 (9.15)
Median	53.00	47.50
25th, 75th Percentile	47.00, 56.00	40.00, 55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	39.0, 56.0	23.0, 56.0
Change from baseline to Week 52 ^a		
n	19	17
Mean (SD)	2.58 (4.32)	0.76 (7.91)
Median	0.00	4.00
25th, 75th Percentile	0.00, 4.00	-1.00, 6.00
Min, Max	-2.0, 14.0	-18.0, 10.0
Difference in change from baseline (95%CI)		-1.81 (-6.27, 2.64)
P-value ^b		0.4091
Hedges'g (95% CI) ^c		-0.28 (-0.94, 0.38)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
WeeFIM : Self-Care Score		
Baseline		
n	17	13
Mean (SD)	47.88 (8.22)	44.15 (10.63)
Median	48.00	49.00
25th, 75th Percentile	42.00, 56.00	34.00, 54.00
Min, Max	30.0, 56.0	30.0, 56.0
Week 26		
n	16	14
Mean (SD)	50.50 (7.01)	46.50 (8.67)
Median	53.00	48.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	48.00, 56.00	40.00, 56.00
Min, Max	30.0, 56.0	31.0, 56.0
Change from baseline to Week 26 ^a		
n	15	13
Mean (SD)	1.53 (3.50)	2.08 (2.81)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.00	0.00, 4.00
Min, Max	-2.0, 10.0	-2.0, 8.0
Week 52		
n	17	13
Mean (SD)	50.65 (7.58)	45.54 (8.88)
Median	54.00	45.00
25th, 75th Percentile	48.00, 56.00	39.00, 55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 56.0	33.0, 56.0
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	2.38 (4.70)	2.17 (2.82)
Median	0.00	2.00
25th, 75th Percentile	0.00, 3.00	0.50, 4.00
Min, Max	-1.0, 18.0	-4.0, 7.0
Difference in change from baseline (95%CI)		-0.21 (-3.36, 2.94)
P-value ^b		0.8930
Hedges'g (95% CI) ^c		-0.05 (-0.80, 0.70)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
WeeFIM : Self-Care Score		
Baseline		
n	24	26
Mean (SD)	45.04 (8.35)	45.38 (9.49)
Median	44.50	47.00
25th, 75th Percentile	40.00, 51.50	40.00, 53.00
Min, Max	22.0, 56.0	24.0, 56.0
Week 26		
n	23	26
Mean (SD)	46.43 (8.64)	47.12 (7.83)
Median	48.00	48.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.00, 53.00	42.00, 54.00
Min, Max	23.0, 56.0	31.0, 56.0
Change from baseline to Week 26 ^a		
n	23	25
Mean (SD)	1.61 (5.86)	1.76 (4.82)
Median	0.00	0.00
25th, 75th Percentile	0.00, 8.00	0.00, 4.00
Min, Max	-10.0, 13.0	-11.0, 15.0
Week 52		
n	24	26
Mean (SD)	46.83 (7.39)	47.92 (7.55)
Median	49.00	49.00
25th, 75th Percentile	42.00, 52.00	43.00, 56.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 56.0	33.0, 56.0
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	1.79 (5.83)	2.52 (4.38)
Median	0.00	2.00
25th, 75th Percentile	-2.00, 3.50	0.00, 6.00
Min, Max	-6.0, 18.0	-8.0, 11.0
Difference in change from baseline (95%CI)		0.73 (-2.23, 3.68)
P-value ^b		0.6224
Hedges'g (95% CI) ^c		0.14 (-0.42, 0.70)
P-value for interaction term, treatment *[Baseline AGV]		0.5513

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
WeeFIM : Self-Care Score		
Baseline		
n	40	42
Mean (SD)	45.93 (8.57)	45.69 (8.78)
Median	48.00	48.50
25th, 75th Percentile	39.50, 53.50	36.00, 53.00
Min, Max	22.0, 56.0	29.0, 56.0
Week 26		
n	39	43
Mean (SD)	47.49 (7.98)	47.12 (8.53)
Median	49.00	50.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	42.00, 56.00	40.00, 56.00
Min, Max	23.0, 56.0	31.0, 56.0
Change from baseline to Week 26 ^a		
n	38	40
Mean (SD)	1.24 (4.47)	1.70 (4.12)
Median	0.00	1.50
25th, 75th Percentile	0.00, 3.00	0.00, 4.50
Min, Max	-10.0, 10.0	-17.0, 10.0
Week 52		
n	41	43
Mean (SD)	48.46 (7.58)	47.42 (7.57)
Median	49.00	48.00
25th, 75th Percentile	42.00, 56.00	40.00, 55.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 56.0	33.0, 56.0
Change from baseline to Week 52 ^a		
n	40	40
Mean (SD)	2.35 (5.19)	2.23 (4.85)
Median	0.00	2.00
25th, 75th Percentile	0.00, 4.00	0.00, 5.50
Min, Max	-6.0, 18.0	-12.0, 10.0
Difference in change from baseline (95%CI)		-0.13 (-2.36, 2.11)
P-value ^b		0.9117
Hedges'g (95% CI) ^c		-0.02 (-0.46, 0.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

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Table 14.2.9.1.2.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
WeeFIM : Self-Care Score		
Baseline		
n	20	15
Mean (SD)	48.35 (7.82)	44.60 (10.52)
Median	51.00	47.00
25th, 75th Percentile	44.00, 55.00	36.00, 53.00
Min, Max	30.0, 56.0	24.0, 56.0
Week 26		
n	19	14
Mean (SD)	49.42 (7.97)	44.43 (9.65)
Median	54.00	46.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.007_qs_sum_ovrtm_weef_sel_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	46.00, 56.00	42.00, 51.00
Min, Max	30.0, 56.0	21.0, 56.0
Change from baseline to Week 26 ^a		
n	19	14
Mean (SD)	1.00 (5.21)	0.43 (8.51)
Median	0.00	0.00
25th, 75th Percentile	-2.00, 2.00	-2.00, 6.00
Min, Max	-10.0, 13.0	-20.0, 15.0
Week 52		
n	19	14
Mean (SD)	50.32 (6.58)	44.93 (10.32)
Median	51.00	48.00
25th, 75th Percentile	47.00, 56.00	35.00, 53.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.007_qs_sum_ovrtm_weef_sel_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.2.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Self Care Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 56.0	23.0, 56.0
Change from baseline to Week 52 ^a		
n	19	14
Mean (SD)	1.89 (4.75)	0.93 (7.04)
Median	0.00	1.50
25th, 75th Percentile	0.00, 3.00	-3.00, 6.00
Min, Max	-4.0, 15.0	-18.0, 11.0
Difference in change from baseline (95%CI)		-0.97 (-5.15, 3.22)
P-value ^b		0.6408
Hedges'g (95% CI) ^c		-0.16 (-0.85, 0.53)
P-value for interaction term, treatment * [Ethnicity]		0.7024

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-56: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.002.007_qs_sum_ovrtm_weef_sel_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
WeeFIM : Mobility Score		
Baseline		
n	33	28
Mean (SD)	31.88 (2.96)	30.71 (3.43)
Median	33.00	32.00
25th, 75th Percentile	30.00, 34.00	28.00, 33.50
Min, Max	23.0, 35.0	21.0, 35.0
Week 26		
n	31	29
Mean (SD)	32.10 (2.61)	32.48 (2.67)
Median	33.00	33.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.003.001_qs_sum_ovrtm_weef_mob_sex_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 35.00	32.00, 34.00
Min, Max	27.0, 35.0	24.0, 35.0
Change from baseline to Week 26 ^a		
n	31	26
Mean (SD)	0.16 (2.61)	1.85 (2.48)
Median	0.00	1.00
25th, 75th Percentile	0.00, 0.00	0.00, 4.00
Min, Max	-7.0, 6.0	-2.0, 7.0
Week 52		
n	32	30
Mean (SD)	32.03 (3.23)	32.17 (2.18)
Median	33.00	32.00
25th, 75th Percentile	30.00, 34.50	31.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.003.001_qs_sum_ovrtm_weef_mob_sex_301_fas.pdf+rtf

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Table 14.2.9.1.3.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	22.0, 35.0	27.0, 35.0
Change from baseline to Week 52 ^a		
n	32	27
Mean (SD)	0.00 (2.42)	1.52 (2.72)
Median	0.00	1.00
25th, 75th Percentile	0.00, 0.00	0.00, 3.00
Min, Max	-8.0, 6.0	-5.0, 7.0
Difference in change from baseline (95%CI)		1.52 (0.18, 2.86)
P-value ^b		0.0272
Hedges'g (95% CI) ^c		0.58 (0.06, 1.11)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
WeeFIM : Mobility Score		
Baseline		
n	27	29
Mean (SD)	30.26 (5.67)	31.24 (4.21)
Median	32.00	33.00
25th, 75th Percentile	29.00, 34.00	29.00, 35.00
Min, Max	12.0, 35.0	20.0, 35.0
Week 26		
n	27	28
Mean (SD)	31.19 (5.01)	31.79 (3.98)
Median	33.00	34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.00, 35.00	30.00, 35.00
Min, Max	18.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	26	28
Mean (SD)	0.92 (3.98)	0.57 (2.56)
Median	0.00	0.00
25th, 75th Percentile	0.00, 3.00	0.00, 1.50
Min, Max	-12.0, 13.0	-5.0, 7.0
Week 52		
n	28	27
Mean (SD)	31.11 (4.37)	31.22 (4.09)
Median	33.00	33.00
25th, 75th Percentile	29.00, 34.50	29.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	27	27
Mean (SD)	0.78 (4.77)	0.15 (3.13)
Median	0.00	0.00
25th, 75th Percentile	-1.00, 3.00	0.00, 2.00
Min, Max	-13.0, 16.0	-8.0, 6.0
Difference in change from baseline (95%CI)		-0.63 (-2.84, 1.58)
P-value ^b		0.5692
Hedges'g (95% CI) ^c		-0.15 (-0.69, 0.38)
P-value for interaction term, treatment * [Sex]		0.0918

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
WeeFIM : Mobility Score		
Baseline		
n	24	31
Mean (SD)	30.17 (3.61)	29.87 (4.39)
Median	30.00	31.00
25th, 75th Percentile	29.00, 33.00	27.00, 34.00
Min, Max	19.0, 34.0	20.0, 35.0
Week 26		
n	24	30
Mean (SD)	30.29 (3.85)	31.07 (3.56)
Median	31.00	32.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	28.00, 33.00	30.00, 34.00
Min, Max	20.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	24	30
Mean (SD)	0.13 (3.83)	1.33 (2.94)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.50	0.00, 3.00
Min, Max	-12.0, 6.0	-4.0, 7.0
Week 52		
n	24	31
Mean (SD)	29.96 (4.25)	30.81 (3.73)
Median	30.50	32.00
25th, 75th Percentile	28.00, 33.50	28.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 34.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	24	31
Mean (SD)	-0.21 (3.64)	0.94 (3.63)
Median	0.00	1.00
25th, 75th Percentile	-0.50, 1.00	0.00, 4.00
Min, Max	-13.0, 6.0	-8.0, 7.0
Difference in change from baseline (95%CI)		1.14 (-0.84, 3.13)
P-value ^b		0.2522
Hedges'g (95% CI) ^c		0.31 (-0.23, 0.84)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
WeeFIM : Mobility Score		
Baseline		
n	24	16
Mean (SD)	30.75 (5.48)	31.56 (2.53)
Median	32.50	32.00
25th, 75th Percentile	29.50, 35.00	29.00, 33.50
Min, Max	12.0, 35.0	27.0, 35.0
Week 26		
n	22	16
Mean (SD)	31.91 (4.31)	32.81 (3.15)
Median	33.50	34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 35.00	32.00, 34.50
Min, Max	18.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	1.14 (3.38)	1.27 (2.40)
Median	0.00	1.00
25th, 75th Percentile	0.00, 1.00	0.00, 3.00
Min, Max	-3.0, 13.0	-5.0, 5.0
Week 52		
n	23	16
Mean (SD)	31.96 (3.51)	32.31 (2.06)
Median	33.00	32.00
25th, 75th Percentile	30.00, 35.00	31.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	22.0, 35.0	29.0, 35.0
Change from baseline to Week 52 ^a		
n	23	15
Mean (SD)	1.04 (4.48)	0.80 (2.27)
Median	0.00	1.00
25th, 75th Percentile	0.00, 1.00	0.00, 3.00
Min, Max	-8.0, 16.0	-4.0, 4.0
Difference in change from baseline (95%CI)		-0.24 (-2.48, 2.00)
P-value ^b		0.8266
Hedges'g (95% CI) ^c		-0.06 (-0.71, 0.59)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
WeeFIM : Mobility Score		
Baseline		
n	12	10
Mean (SD)	33.92 (2.07)	33.50 (2.07)
Median	35.00	34.50
25th, 75th Percentile	34.00, 35.00	33.00, 35.00
Min, Max	28.0, 35.0	29.0, 35.0
Week 26		
n	12	11
Mean (SD)	34.00 (1.54)	34.09 (1.81)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	33.00, 35.00	34.00, 35.00
Min, Max	30.0, 35.0	29.0, 35.0
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	0.09 (1.30)	0.56 (1.42)
Median	0.00	0.00
25th, 75th Percentile	-1.00, 0.00	0.00, 1.00
Min, Max	-1.0, 3.0	-1.0, 4.0
Week 52		
n	13	10
Mean (SD)	34.00 (1.41)	33.60 (2.07)
Median	34.00	35.00
25th, 75th Percentile	34.00, 35.00	32.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.003.002_qs_sum_ovrtm_weef_mob_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

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Table 14.2.9.1.3.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 35.0	29.0, 35.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	0.17 (1.27)	0.50 (0.76)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 1.00
Min, Max	-2.0, 3.0	0.0, 2.0
Difference in change from baseline (95%CI)		0.33 (-0.72, 1.39)
P-value ^b		0.5141
Hedges'g (95% CI) ^c		0.29 (-0.61, 1.19)
P-value for interaction term, treatment *[Age at Baseline]		0.6300

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
WeeFIM : Mobility Score		
Baseline		
n	47	46
Mean (SD)	30.83 (4.27)	30.50 (3.95)
Median	32.00	32.00
25th, 75th Percentile	29.00, 34.00	28.00, 34.00
Min, Max	12.0, 35.0	20.0, 35.0
Week 26		
n	45	46
Mean (SD)	31.18 (3.98)	31.78 (3.54)
Median	32.00	33.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 34.00	31.00, 34.00
Min, Max	18.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	44	44
Mean (SD)	0.34 (3.19)	1.36 (2.79)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.00	0.00, 3.00
Min, Max	-12.0, 6.0	-5.0, 7.0
Week 52		
n	47	47
Mean (SD)	31.11 (3.95)	31.40 (3.29)
Median	33.00	32.00
25th, 75th Percentile	30.00, 34.00	30.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	46	45
Mean (SD)	0.15 (3.93)	0.98 (3.16)
Median	0.00	1.00
25th, 75th Percentile	-1.00, 1.00	0.00, 2.00
Min, Max	-13.0, 16.0	-8.0, 7.0
Difference in change from baseline (95%CI)		0.83 (-0.66, 2.31)
P-value ^b		0.2727
Hedges'g (95% CI) ^c		0.23 (-0.18, 0.64)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
WeeFIM : Mobility Score		
Baseline		
n	13	11
Mean (SD)	32.31 (4.96)	33.00 (2.49)
Median	35.00	33.00
25th, 75th Percentile	34.00, 35.00	32.00, 35.00
Min, Max	22.0, 35.0	28.0, 35.0
Week 26		
n	13	11
Mean (SD)	33.38 (3.18)	33.64 (1.96)
Median	35.00	34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	33.00, 35.00	33.00, 35.00
Min, Max	24.0, 35.0	29.0, 35.0
Change from baseline to Week 26 ^a		
n	13	10
Mean (SD)	1.08 (3.71)	0.40 (1.07)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 1.00
Min, Max	-1.0, 13.0	-1.0, 3.0
Week 52		
n	13	10
Mean (SD)	33.38 (2.63)	33.20 (2.57)
Median	35.00	35.00
25th, 75th Percentile	34.00, 35.00	31.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 35.0	29.0, 35.0
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	1.08 (2.56)	0.11 (1.90)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-1.0, 8.0	-4.0, 3.0
Difference in change from baseline (95%CI)		-0.97 (-3.07, 1.13)
P-value ^b		0.3489
Hedges'g (95% CI) ^c		-0.40 (-1.25, 0.46)
P-value for interaction term, treatment * [Baseline Tanner Stage]		0.2727

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
WeeFIM : Mobility Score		
Baseline		
n	10	14
Mean (SD)	30.60 (4.60)	31.00 (4.31)
Median	31.50	32.50
25th, 75th Percentile	29.00, 34.00	29.00, 34.00
Min, Max	19.0, 35.0	20.0, 35.0
Week 26		
n	10	13
Mean (SD)	28.80 (5.12)	31.00 (5.00)
Median	29.00	34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	27.00, 34.00	30.00, 34.00
Min, Max	20.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	-1.80 (4.37)	0.08 (2.84)
Median	0.00	0.00
25th, 75th Percentile	-2.00, 1.00	-1.50, 2.00
Min, Max	-12.0, 2.0	-5.0, 5.0
Week 52		
n	10	13
Mean (SD)	28.90 (4.98)	31.08 (3.62)
Median	30.00	32.00
25th, 75th Percentile	27.00, 33.00	30.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 34.0	23.0, 35.0
Change from baseline to Week 52 ^a		
n	10	12
Mean (SD)	-1.70 (4.40)	0.42 (2.61)
Median	-1.00	0.50
25th, 75th Percentile	-2.00, 1.00	-1.00, 1.50
Min, Max	-13.0, 3.0	-4.0, 5.0
Difference in change from baseline (95%CI)		2.12 (-1.03, 5.27)
P-value ^b		0.1765
Hedges'g (95% CI) ^c		0.58 (-0.29, 1.43)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
WeeFIM : Mobility Score		
Baseline		
n	23	18
Mean (SD)	31.22 (5.35)	29.33 (3.12)
Median	33.00	28.50
25th, 75th Percentile	29.00, 35.00	27.00, 33.00
Min, Max	12.0, 35.0	23.0, 34.0
Week 26		
n	22	18
Mean (SD)	32.05 (3.97)	31.72 (2.63)
Median	33.00	32.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.00, 35.00	30.00, 34.00
Min, Max	18.0, 35.0	26.0, 35.0
Change from baseline to Week 26 ^a		
n	21	18
Mean (SD)	0.67 (2.50)	2.39 (2.43)
Median	0.00	2.00
25th, 75th Percentile	0.00, 2.00	1.00, 4.00
Min, Max	-5.0, 6.0	-2.0, 7.0
Week 52		
n	23	18
Mean (SD)	32.52 (3.22)	31.44 (3.01)
Median	34.00	32.00
25th, 75th Percentile	31.00, 35.00	29.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	23.0, 35.0	24.0, 35.0
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	1.09 (3.77)	2.11 (1.94)
Median	0.00	2.00
25th, 75th Percentile	0.00, 1.00	1.00, 4.00
Min, Max	-4.0, 16.0	-1.0, 6.0
Difference in change from baseline (95%CI)		1.02 (-0.86, 2.90)
P-value ^b		0.2774
Hedges'g (95% CI) ^c		0.32 (-0.31, 0.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -5 to <= -4		
WeeFIM : Mobility Score		
Baseline		
n	19	21
Mean (SD)	31.11 (3.91)	31.95 (3.90)
Median	32.00	34.00
25th, 75th Percentile	30.00, 34.00	31.00, 35.00
Min, Max	22.0, 35.0	21.0, 35.0
Week 26		
n	18	21
Mean (SD)	32.50 (2.90)	33.00 (2.53)
Median	33.00	33.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	32.00, 35.00	32.00, 35.00
Min, Max	24.0, 35.0	24.0, 35.0
Change from baseline to Week 26 ^a		
n	18	20
Mean (SD)	1.56 (3.40)	1.15 (2.30)
Median	0.00	0.00
25th, 75th Percentile	0.00, 3.00	0.00, 2.00
Min, Max	-1.0, 13.0	-2.0, 7.0
Week 52		
n	19	21
Mean (SD)	32.26 (2.54)	32.29 (2.57)
Median	33.00	32.00
25th, 75th Percentile	30.00, 35.00	31.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 35.0	27.0, 35.0
Change from baseline to Week 52 ^a		
n	19	20
Mean (SD)	1.16 (2.69)	0.50 (3.41)
Median	0.00	0.00
25th, 75th Percentile	0.00, 2.00	0.00, 2.00
Min, Max	-3.0, 8.0	-7.0, 7.0
Difference in change from baseline (95%CI)		-0.66 (-2.66, 1.34)
P-value ^b		0.5094
Hedges'g (95% CI) ^c		-0.21 (-0.84, 0.42)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.003.005_qs_sum_ovrtm_weef_mob_bhgt_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
WeeFIM : Mobility Score		
Baseline		
n	8	4
Mean (SD)	31.75 (2.76)	33.25 (2.36)
Median	31.50	34.00
25th, 75th Percentile	29.00, 34.50	31.50, 35.00
Min, Max	29.0, 35.0	30.0, 35.0
Week 26		
n	8	5
Mean (SD)	32.38 (2.88)	33.00 (3.46)
Median	33.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	30.50, 35.00	33.00, 35.00
Min, Max	27.0, 35.0	27.0, 35.0
Change from baseline to Week 26 ^a		
n	8	4
Mean (SD)	0.63 (2.45)	-0.75 (1.50)
Median	0.00	0.00
25th, 75th Percentile	-0.50, 1.00	-1.50, 0.00
Min, Max	-2.0, 6.0	-3.0, 0.0
Week 52		
n	8	5
Mean (SD)	30.75 (5.06)	32.00 (5.66)
Median	33.50	35.00
25th, 75th Percentile	26.50, 34.50	33.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	22.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	8	4
Mean (SD)	-1.00 (3.74)	-2.00 (4.00)
Median	0.00	0.00
25th, 75th Percentile	-2.50, 0.00	-4.00, 0.00
Min, Max	-8.0, 5.0	-8.0, 0.0
Difference in change from baseline (95%CI)		-1.00 (-6.21, 4.21)
P-value ^b		0.6782
Hedges'g (95% CI) ^c		-0.24 (-1.44, 0.97)
P-value for interaction term, treatment * [Baseline Height Z-score]		0.3384

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
WeeFIM : Mobility Score		
Baseline		
n	19	18
Mean (SD)	31.95 (3.87)	31.78 (3.39)
Median	33.00	33.00
25th, 75th Percentile	30.00, 35.00	31.00, 34.00
Min, Max	22.0, 35.0	25.0, 35.0
Week 26		
n	19	17
Mean (SD)	31.79 (3.51)	32.76 (3.05)
Median	34.00	34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.00, 35.00	32.00, 35.00
Min, Max	24.0, 35.0	24.0, 35.0
Change from baseline to Week 26 ^a		
n	19	16
Mean (SD)	-0.16 (2.43)	1.31 (2.65)
Median	0.00	1.00
25th, 75th Percentile	-1.00, 1.00	0.00, 3.00
Min, Max	-7.0, 5.0	-4.0, 7.0
Week 52		
n	19	18
Mean (SD)	32.32 (3.16)	32.00 (3.01)
Median	34.00	32.50
25th, 75th Percentile	30.00, 35.00	31.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	25.0, 35.0	23.0, 35.0
Change from baseline to Week 52 ^a		
n	19	17
Mean (SD)	0.37 (2.61)	0.41 (3.00)
Median	0.00	1.00
25th, 75th Percentile	0.00, 1.00	-1.00, 1.00
Min, Max	-4.0, 8.0	-5.0, 7.0
Difference in change from baseline (95%CI)		0.04 (-1.86, 1.94)
P-value ^b		0.9633
Hedges'g (95% CI) ^c		0.02 (-0.64, 0.67)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
WeeFIM : Mobility Score		
Baseline		
n	17	13
Mean (SD)	32.65 (2.76)	30.00 (4.83)
Median	34.00	29.00
25th, 75th Percentile	30.00, 35.00	27.00, 34.00
Min, Max	27.0, 35.0	21.0, 35.0
Week 26		
n	16	14
Mean (SD)	33.81 (1.72)	31.64 (4.48)
Median	35.00	33.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	33.00, 35.00	30.00, 35.00
Min, Max	30.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	15	13
Mean (SD)	0.87 (1.88)	1.38 (3.23)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.00	0.00, 3.00
Min, Max	-1.0, 6.0	-5.0, 7.0
Week 52		
n	17	13
Mean (SD)	33.41 (1.80)	31.08 (3.57)
Median	34.00	32.00
25th, 75th Percentile	33.00, 35.00	28.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	30.0, 35.0	24.0, 35.0
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	0.44 (1.63)	1.42 (3.50)
Median	0.00	1.00
25th, 75th Percentile	0.00, 0.00	0.00, 4.00
Min, Max	-2.0, 5.0	-7.0, 6.0
Difference in change from baseline (95%CI)		0.98 (-1.35, 3.31)
P-value ^b		0.3837
Hedges'g (95% CI) ^c		0.37 (-0.39, 1.12)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
WeeFIM : Mobility Score		
Baseline		
n	24	26
Mean (SD)	29.46 (5.30)	30.92 (3.58)
Median	30.50	32.00
25th, 75th Percentile	28.50, 33.00	29.00, 33.00
Min, Max	12.0, 35.0	20.0, 35.0
Week 26		
n	23	26
Mean (SD)	30.09 (4.63)	32.00 (2.91)
Median	31.00	32.50

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.00, 33.00	31.00, 34.00
Min, Max	18.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	23	25
Mean (SD)	0.83 (4.47)	1.00 (2.24)
Median	0.00	0.00
25th, 75th Percentile	0.00, 3.00	0.00, 2.00
Min, Max	-12.0, 13.0	-3.0, 6.0
Week 52		
n	24	26
Mean (SD)	29.75 (4.53)	31.85 (3.29)
Median	31.00	32.50
25th, 75th Percentile	27.50, 33.50	30.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	0.29 (5.19)	0.84 (2.79)
Median	0.00	0.00
25th, 75th Percentile	-1.00, 1.50	0.00, 2.00
Min, Max	-13.0, 16.0	-8.0, 6.0
Difference in change from baseline (95%CI)		0.55 (-1.88, 2.98)
P-value ^b		0.6498
Hedges'g (95% CI) ^c		0.13 (-0.43, 0.69)
P-value for interaction term, treatment *[Baseline AGV]		0.8618

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
WeeFIM : Mobility Score		
Baseline		
n	40	42
Mean (SD)	30.98 (4.88)	31.24 (3.53)
Median	32.00	32.00
25th, 75th Percentile	29.50, 34.50	29.00, 34.00
Min, Max	12.0, 35.0	21.0, 35.0
Week 26		
n	39	43
Mean (SD)	31.18 (4.45)	32.30 (3.02)
Median	33.00	33.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.00, 35.00	31.00, 35.00
Min, Max	18.0, 35.0	24.0, 35.0
Change from baseline to Week 26 ^a		
n	38	40
Mean (SD)	0.29 (3.18)	1.08 (2.39)
Median	0.00	1.00
25th, 75th Percentile	0.00, 2.00	0.00, 2.50
Min, Max	-12.0, 6.0	-4.0, 7.0
Week 52		
n	41	43
Mean (SD)	31.10 (4.21)	31.56 (3.38)
Median	33.00	32.00
25th, 75th Percentile	30.00, 35.00	29.00, 34.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	40	40
Mean (SD)	0.08 (4.23)	0.40 (3.07)
Median	0.00	0.50
25th, 75th Percentile	-1.00, 0.50	0.00, 2.00
Min, Max	-13.0, 16.0	-8.0, 7.0
Difference in change from baseline (95%CI)		0.33 (-1.32, 1.97)
P-value ^b		0.6954
Hedges'g (95% CI) ^c		0.09 (-0.35, 0.53)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.3.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
WeeFIM : Mobility Score		
Baseline		
n	20	15
Mean (SD)	31.50 (3.43)	30.27 (4.62)
Median	33.00	32.00
25th, 75th Percentile	29.00, 34.00	27.00, 34.00
Min, Max	22.0, 35.0	20.0, 35.0
Week 26		
n	19	14
Mean (SD)	32.68 (2.19)	31.64 (4.34)
Median	33.00	33.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.003.007_qs_sum_ovrtm_weef_mob_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.00, 35.00	31.00, 34.00
Min, Max	28.0, 35.0	22.0, 35.0
Change from baseline to Week 26 ^a		
n	19	14
Mean (SD)	0.95 (3.57)	1.50 (3.13)
Median	0.00	0.50
25th, 75th Percentile	0.00, 1.00	0.00, 5.00
Min, Max	-5.0, 13.0	-5.0, 6.0
Week 52		
n	19	14
Mean (SD)	32.68 (2.47)	32.21 (2.78)
Median	34.00	32.50
25th, 75th Percentile	30.00, 34.00	31.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.3.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Mobility Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	27.0, 35.0	25.0, 35.0
Change from baseline to Week 52 ^a		
n	19	14
Mean (SD)	0.95 (2.01)	2.07 (2.43)
Median	0.00	1.00
25th, 75th Percentile	0.00, 1.00	0.00, 4.00
Min, Max	-1.0, 6.0	-1.0, 6.0
Difference in change from baseline (95%CI)		1.12 (-0.46, 2.70)
P-value ^b		0.1567
Hedges'g (95% CI) ^c		0.50 (-0.21, 1.20)
P-value for interaction term, treatment * [Ethnicity]		0.5676

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Male		
WeeFIM : Cognitive Score		
Baseline		
n	33	28
Mean (SD)	32.67 (5.12)	32.32 (5.03)
Median	35.00	34.00
25th, 75th Percentile	32.00, 35.00	32.50, 35.00
Min, Max	11.0, 35.0	11.0, 35.0
Week 26		
n	31	29
Mean (SD)	33.39 (4.86)	32.86 (3.95)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	32.00, 35.00
Min, Max	11.0, 35.0	18.0, 35.0
Change from baseline to Week 26 ^a		
n	31	26
Mean (SD)	0.61 (1.56)	0.58 (3.96)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 2.00
Min, Max	-2.0, 5.0	-8.0, 11.0
Week 52		
n	32	30
Mean (SD)	32.06 (6.06)	32.13 (4.24)
Median	35.00	35.00
25th, 75th Percentile	32.00, 35.00	31.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	11.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	32	27
Mean (SD)	-0.53 (3.06)	-0.41 (4.33)
Median	0.00	0.00
25th, 75th Percentile	-0.50, 0.00	0.00, 0.00
Min, Max	-10.0, 8.0	-11.0, 11.0
Difference in change from baseline (95%CI)		0.12 (-1.81, 2.06)
P-value ^b		0.8984
Hedges'g (95% CI) ^c		0.03 (-0.48, 0.55)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Female		
WeeFIM : Cognitive Score		
Baseline		
n	27	29
Mean (SD)	32.70 (3.37)	34.52 (1.60)
Median	35.00	35.00
25th, 75th Percentile	29.00, 35.00	35.00, 35.00
Min, Max	23.0, 35.0	28.0, 35.0
Week 26		
n	27	28
Mean (SD)	32.96 (4.31)	34.50 (1.17)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.00, 35.00	35.00, 35.00
Min, Max	19.0, 35.0	31.0, 35.0
Change from baseline to Week 26 ^a		
n	26	28
Mean (SD)	0.19 (3.18)	-0.07 (1.15)
Median	0.00	0.00
25th, 75th Percentile	0.00, 1.00	0.00, 0.00
Min, Max	-7.0, 6.0	-3.0, 4.0
Week 52		
n	28	27
Mean (SD)	31.93 (5.52)	34.15 (3.06)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	35.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.1
Functional Independence Measure For Children (WeeFIM) Over Time by Sex: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Sex Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.0, 35.0	20.0, 35.0
Change from baseline to Week 52 ^a		
n	27	27
Mean (SD)	-0.89 (5.12)	-0.41 (2.55)
Median	0.00	0.00
25th, 75th Percentile	-1.00, 0.00	0.00, 0.00
Min, Max	-17.0, 6.0	-10.0, 6.0
Difference in change from baseline (95%CI)		0.48 (-1.75, 2.71)
P-value ^b		0.6644
Hedges'g (95% CI) ^c		0.12 (-0.42, 0.65)
P-value for interaction term, treatment * [Sex]		0.8068

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 5 to < 8		
WeeFIM : Cognitive Score		
Baseline		
n	24	31
Mean (SD)	31.88 (5.66)	33.74 (2.31)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	33.00, 35.00
Min, Max	11.0, 35.0	27.0, 35.0
Week 26		
n	24	30
Mean (SD)	32.00 (5.81)	33.80 (2.50)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	31.00, 35.00	35.00, 35.00
Min, Max	11.0, 35.0	27.0, 35.0
Change from baseline to Week 26 ^a		
n	24	30
Mean (SD)	0.13 (2.88)	0.10 (2.75)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.50	0.00, 0.00
Min, Max	-7.0, 6.0	-8.0, 8.0
Week 52		
n	24	31
Mean (SD)	30.17 (7.21)	33.48 (2.97)
Median	35.00	35.00
25th, 75th Percentile	26.50, 35.00	33.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	11.0, 35.0	25.0, 35.0
Change from baseline to Week 52 ^a		
n	24	31
Mean (SD)	-1.71 (5.50)	-0.26 (3.19)
Median	0.00	0.00
25th, 75th Percentile	-4.00, 0.00	0.00, 0.00
Min, Max	-17.0, 8.0	-9.0, 8.0
Difference in change from baseline (95%CI)		1.45 (-1.11, 4.01)
P-value ^b		0.2582
Hedges'g (95% CI) ^c		0.33 (-0.21, 0.86)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 8 to < 11		
WeeFIM : Cognitive Score		
Baseline		
n	24	16
Mean (SD)	33.38 (3.03)	33.06 (6.03)
Median	35.00	35.00
25th, 75th Percentile	32.50, 35.00	35.00, 35.00
Min, Max	23.0, 35.0	11.0, 35.0
Week 26		
n	22	16
Mean (SD)	34.09 (3.44)	33.44 (4.34)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	35.00, 35.00
Min, Max	19.0, 35.0	18.0, 35.0
Change from baseline to Week 26 ^a		
n	22	15
Mean (SD)	0.77 (2.22)	0.53 (1.81)
Median	0.00	0.00
25th, 75th Percentile	0.00, 1.00	0.00, 0.00
Min, Max	-4.0, 6.0	0.0, 7.0
Week 52		
n	23	16
Mean (SD)	33.91 (3.00)	33.00 (4.80)
Median	35.00	35.00
25th, 75th Percentile	35.00, 35.00	35.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	22.0, 35.0	20.0, 35.0
Change from baseline to Week 52 ^a		
n	23	15
Mean (SD)	0.61 (2.44)	-0.20 (4.11)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-4.0, 6.0	-10.0, 11.0
Difference in change from baseline (95%CI)		-0.81 (-3.26, 1.64)
P-value ^b		0.4998
Hedges'g (95% CI) ^c		-0.25 (-0.90, 0.41)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
>= 11 to < 15		
WeeFIM : Cognitive Score		
Baseline		
n	12	10
Mean (SD)	32.92 (3.80)	33.10 (3.51)
Median	35.00	35.00
25th, 75th Percentile	32.00, 35.00	32.00, 35.00
Min, Max	24.0, 35.0	24.0, 35.0
Week 26		
n	12	11
Mean (SD)	33.92 (3.18)	33.64 (2.20)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	32.00, 35.00
Min, Max	24.0, 35.0	28.0, 35.0
Change from baseline to Week 26 ^a		
n	11	9
Mean (SD)	0.36 (1.69)	0.22 (4.58)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	-1.00, 0.00
Min, Max	-2.0, 5.0	-6.0, 11.0
Week 52		
n	13	10
Mean (SD)	32.00 (5.82)	32.00 (4.67)
Median	35.00	34.50
25th, 75th Percentile	34.00, 35.00	31.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.004.002_qs_sum_ovrtm_weef_cog_age_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.2
Functional Independence Measure For Children (WeeFIM) Over Time by Age at Baseline: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Age at Baseline Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.0, 35.0	23.0, 35.0
Change from baseline to Week 52 ^a		
n	12	8
Mean (SD)	-1.17 (2.69)	-1.38 (3.89)
Median	0.00	0.00
25th, 75th Percentile	-1.50, 0.00	0.00, 0.00
Min, Max	-9.0, 1.0	-11.0, 0.0
Difference in change from baseline (95%CI)		-0.21 (-3.29, 2.87)
P-value ^b		0.8885
Hedges'g (95% CI) ^c		-0.06 (-0.96, 0.83)
P-value for interaction term, treatment *[Age at Baseline]		0.3606

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: I		
WeeFIM : Cognitive Score		
Baseline		
n	47	46
Mean (SD)	32.81 (4.43)	33.11 (4.20)
Median	35.00	35.00
25th, 75th Percentile	32.00, 35.00	33.00, 35.00
Min, Max	11.0, 35.0	11.0, 35.0
Week 26		
n	45	46
Mean (SD)	33.31 (4.46)	33.46 (3.31)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.004.003_qs_sum_ovrtm_weef_cog_tan_301_fas.pdf+rtf

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Table 14.2.9.1.4.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	33.00, 35.00
Min, Max	11.0, 35.0	18.0, 35.0
Change from baseline to Week 26 ^a		
n	44	44
Mean (SD)	0.39 (2.40)	0.39 (3.13)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.50
Min, Max	-7.0, 6.0	-8.0, 11.0
Week 52		
n	47	47
Mean (SD)	31.91 (6.00)	32.72 (4.13)
Median	35.00	35.00
25th, 75th Percentile	31.00, 35.00	32.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	11.0, 35.0	20.0, 35.0
Change from baseline to Week 52 ^a		
n	46	45
Mean (SD)	-0.91 (4.50)	-0.49 (3.86)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-17.0, 8.0	-11.0, 11.0
Difference in change from baseline (95%CI)		0.42 (-1.32, 2.17)
P-value ^b		0.6309
Hedges'g (95% CI) ^c		0.10 (-0.31, 0.51)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Tanner Stage: > I		
WeeFIM : Cognitive Score		
Baseline		
n	13	11
Mean (SD)	32.23 (4.36)	34.82 (0.60)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	35.00, 35.00
Min, Max	23.0, 35.0	33.0, 35.0
Week 26		
n	13	11
Mean (SD)	32.77 (5.13)	34.55 (0.93)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	34.00, 35.00
Min, Max	19.0, 35.0	32.0, 35.0
Change from baseline to Week 26 ^a		
n	13	10
Mean (SD)	0.54 (2.57)	-0.40 (0.97)
Median	0.00	0.00
25th, 75th Percentile	0.00, 1.00	0.00, 0.00
Min, Max	-4.0, 6.0	-3.0, 0.0
Week 52		
n	13	10
Mean (SD)	32.31 (5.07)	34.80 (0.63)
Median	35.00	35.00
25th, 75th Percentile	34.00, 35.00	35.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.3
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Tanner: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Tanner Stage Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	22.0, 35.0	33.0, 35.0
Change from baseline to Week 52 ^a		
n	13	9
Mean (SD)	0.08 (2.10)	0.00 (0.00)
Median	0.00	0.00
25th, 75th Percentile	-1.00, 0.00	0.00, 0.00
Min, Max	-3.0, 6.0	0.0, 0.0
Difference in change from baseline (95%CI)		-0.08 (-1.35, 1.19)
P-value ^b		0.8971
Hedges'g (95% CI) ^c		-0.05 (-0.89, 0.81)
P-value for interaction term, treatment *[Baseline Tanner Stage]		0.7878

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= -6		
WeeFIM : Cognitive Score		
Baseline		
n	10	14
Mean (SD)	33.00 (2.79)	34.29 (1.54)
Median	35.00	35.00
25th, 75th Percentile	31.00, 35.00	35.00, 35.00
Min, Max	28.0, 35.0	30.0, 35.0
Week 26		
n	10	13
Mean (SD)	33.60 (2.72)	34.23 (1.48)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.00, 35.00	35.00, 35.00
Min, Max	28.0, 35.0	31.0, 35.0
Change from baseline to Week 26 ^a		
n	10	12
Mean (SD)	0.60 (3.37)	-0.42 (1.24)
Median	0.00	0.00
25th, 75th Percentile	0.00, 2.00	0.00, 0.00
Min, Max	-7.0, 6.0	-3.0, 1.0
Week 52		
n	10	13
Mean (SD)	31.60 (6.17)	33.00 (4.34)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	34.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.0, 35.0	20.0, 35.0
Change from baseline to Week 52 ^a		
n	10	12
Mean (SD)	-1.40 (6.52)	-1.67 (3.28)
Median	0.00	0.00
25th, 75th Percentile	0.00, 2.00	-2.00, 0.00
Min, Max	-17.0, 6.0	-10.0, 0.0
Difference in change from baseline (95%CI)		-0.27 (-5.18, 4.64)
P-value ^b		0.9083
Hedges'g (95% CI) ^c		-0.05 (-0.89, 0.79)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -6 to <= -5		
WeeFIM : Cognitive Score		
Baseline		
n	23	18
Mean (SD)	33.04 (3.43)	31.61 (6.23)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	28.00, 35.00
Min, Max	24.0, 35.0	11.0, 35.0
Week 26		
n	22	18
Mean (SD)	33.82 (3.40)	32.94 (4.47)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	33.00, 35.00
Min, Max	23.0, 35.0	18.0, 35.0
Change from baseline to Week 26 ^a		
n	21	18
Mean (SD)	0.52 (2.02)	1.33 (4.10)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 2.00
Min, Max	-4.0, 6.0	-8.0, 11.0
Week 52		
n	23	18
Mean (SD)	32.65 (5.02)	32.39 (4.47)
Median	35.00	35.00
25th, 75th Percentile	35.00, 35.00	33.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	22	18
Mean (SD)	-0.41 (3.05)	0.78 (4.08)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-9.0, 6.0	-8.0, 11.0
Difference in change from baseline (95%CI)		1.19 (-1.10, 3.47)
P-value ^b		0.2992
Hedges'g (95% CI) ^c		0.33 (-0.30, 0.95)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<hr/>		
> -5 to <= -4		
WeeFIM : Cognitive Score		
Baseline		
n	19	21
Mean (SD)	31.74 (6.43)	34.19 (1.44)
Median	35.00	35.00
25th, 75th Percentile	31.00, 35.00	34.00, 35.00
Min, Max	11.0, 35.0	30.0, 35.0
Week 26		
n	18	21
Mean (SD)	31.61 (6.90)	34.00 (2.17)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.00, 35.00	35.00, 35.00
Min, Max	11.0, 35.0	27.0, 35.0
Change from baseline to Week 26 ^a		
n	18	20
Mean (SD)	-0.06 (2.51)	0.00 (1.84)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.50
Min, Max	-6.0, 6.0	-7.0, 2.0
Week 52		
n	19	21
Mean (SD)	31.26 (6.77)	34.00 (2.45)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	35.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	11.0, 35.0	25.0, 35.0
Change from baseline to Week 52 ^a		
n	19	20
Mean (SD)	-0.47 (3.99)	-0.20 (2.26)
Median	0.00	0.00
25th, 75th Percentile	-1.00, 0.00	0.00, 0.00
Min, Max	-12.0, 8.0	-9.0, 2.0
Difference in change from baseline (95%CI)		0.27 (-1.87, 2.42)
P-value ^b		0.7955
Hedges'g (95% CI) ^c		0.08 (-0.55, 0.71)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> -4		
WeeFIM : Cognitive Score		
Baseline		
n	8	4
Mean (SD)	33.50 (2.27)	34.75 (0.50)
Median	35.00	35.00
25th, 75th Percentile	32.00, 35.00	34.50, 35.00
Min, Max	29.0, 35.0	34.0, 35.0
Week 26		
n	8	5
Mean (SD)	34.50 (0.93)	33.40 (3.05)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.00, 35.00	34.00, 35.00
Min, Max	33.0, 35.0	28.0, 35.0
Change from baseline to Week 26 ^a		
n	8	4
Mean (SD)	1.00 (2.07)	-1.50 (3.00)
Median	0.00	0.00
25th, 75th Percentile	0.00, 3.00	-3.00, 0.00
Min, Max	-2.0, 4.0	-6.0, 0.0
Week 52		
n	8	5
Mean (SD)	32.38 (5.58)	32.00 (5.10)
Median	35.00	34.00
25th, 75th Percentile	32.50, 35.00	33.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.5
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline Height Z-score: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline Height Z-score Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 35.0	23.0, 35.0
Change from baseline to Week 52 ^a		
n	8	4
Mean (SD)	-1.13 (3.80)	-3.00 (5.35)
Median	0.00	-0.50
25th, 75th Percentile	-1.00, 0.00	-6.00, 0.00
Min, Max	-10.0, 3.0	-11.0, 0.0
Difference in change from baseline (95%CI)		-1.88 (-7.77, 4.02)
P-value ^b		0.4949
Hedges'g (95% CI) ^c		-0.40 (-1.60, 0.82)
P-value for interaction term, treatment *[Baseline Height Z-score]		0.6830

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
<= 3.5 cm/year		
WeeFIM : Cognitive Score		
Baseline		
n	19	18
Mean (SD)	33.84 (2.14)	32.89 (3.16)
Median	35.00	35.00
25th, 75th Percentile	34.00, 35.00	31.00, 35.00
Min, Max	29.0, 35.0	24.0, 35.0
Week 26		
n	19	17
Mean (SD)	34.84 (0.50)	32.76 (2.99)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	31.00, 35.00
Min, Max	33.0, 35.0	27.0, 35.0
Change from baseline to Week 26 ^a		
n	19	16
Mean (SD)	1.00 (1.89)	0.06 (3.89)
Median	0.00	0.00
25th, 75th Percentile	0.00, 1.00	0.00, 0.50
Min, Max	0.0, 6.0	-8.0, 11.0
Week 52		
n	19	18
Mean (SD)	33.74 (4.01)	31.17 (5.15)
Median	35.00	35.00
25th, 75th Percentile	35.00, 35.00	27.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	19.0, 35.0	20.0, 35.0
Change from baseline to Week 52 ^a		
n	19	17
Mean (SD)	-0.11 (2.94)	-2.00 (4.11)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	-3.00, 0.00
Min, Max	-10.0, 6.0	-11.0, 2.0
Difference in change from baseline (95%CI)		-1.89 (-4.30, 0.51)
P-value ^b		0.1180
Hedges'g (95% CI) ^c		-0.52 (-1.19, 0.15)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 3.5 to <= 4.5 cm/year		
WeeFIM : Cognitive Score		
Baseline		
n	17	13
Mean (SD)	32.24 (6.36)	32.69 (6.58)
Median	35.00	35.00
25th, 75th Percentile	35.00, 35.00	33.00, 35.00
Min, Max	11.0, 35.0	11.0, 35.0
Week 26		
n	16	14
Mean (SD)	32.63 (6.39)	33.57 (4.52)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.50, 35.00	35.00, 35.00
Min, Max	11.0, 35.0	18.0, 35.0
Change from baseline to Week 26 ^a		
n	15	13
Mean (SD)	0.07 (1.22)	0.77 (2.05)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-2.0, 4.0	-1.0, 7.0
Week 52		
n	17	13
Mean (SD)	30.76 (7.50)	33.31 (3.61)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	33.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	11.0, 35.0	22.0, 35.0
Change from baseline to Week 52 ^a		
n	16	12
Mean (SD)	-1.56 (3.01)	0.75 (3.28)
Median	0.00	0.00
25th, 75th Percentile	-1.50, 0.00	0.00, 0.00
Min, Max	-9.0, 0.0	-2.0, 11.0
Difference in change from baseline (95%CI)		2.31 (-0.14, 4.77)
P-value ^b		0.0637
Hedges'g (95% CI) ^c		0.72 (-0.06, 1.48)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
> 4.5 cm/year		
WeeFIM : Cognitive Score		
Baseline		
n	24	26
Mean (SD)	32.08 (4.00)	34.19 (2.06)
Median	35.00	35.00
25th, 75th Percentile	29.00, 35.00	35.00, 35.00
Min, Max	22.0, 35.0	27.0, 35.0
Week 26		
n	23	26
Mean (SD)	32.22 (4.76)	34.31 (1.78)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	29.00, 35.00	35.00, 35.00
Min, Max	19.0, 35.0	27.0, 35.0
Change from baseline to Week 26 ^a		
n	23	25
Mean (SD)	0.17 (3.26)	0.08 (2.52)
Median	0.00	0.00
25th, 75th Percentile	0.00, 3.00	0.00, 0.00
Min, Max	-7.0, 6.0	-7.0, 8.0
Week 52		
n	24	26
Mean (SD)	31.50 (5.46)	34.31 (2.09)
Median	35.00	35.00
25th, 75th Percentile	30.00, 35.00	35.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.6
Functional Independence Measure For Children (WeeFIM) Over Time by Baseline AGV Category: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Baseline AGV Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.0, 35.0	25.0, 35.0
Change from baseline to Week 52 ^a		
n	24	25
Mean (SD)	-0.58 (5.37)	0.12 (2.91)
Median	0.00	0.00
25th, 75th Percentile	-1.50, 1.00	0.00, 0.00
Min, Max	-17.0, 8.0	-9.0, 8.0
Difference in change from baseline (95%CI)		0.70 (-1.82, 3.22)
P-value ^b		0.5745
Hedges'g (95% CI) ^c		0.16 (-0.40, 0.72)
P-value for interaction term, treatment *[Baseline AGV]		0.0849

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
White		
WeeFIM : Cognitive Score		
Baseline		
n	40	42
Mean (SD)	32.88 (3.38)	33.33 (4.20)
Median	35.00	35.00
25th, 75th Percentile	31.50, 35.00	33.00, 35.00
Min, Max	22.0, 35.0	11.0, 35.0
Week 26		
n	39	43
Mean (SD)	33.54 (3.35)	33.56 (3.25)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	34.00, 35.00	34.00, 35.00
Min, Max	23.0, 35.0	18.0, 35.0
Change from baseline to Week 26 ^a		
n	38	40
Mean (SD)	0.47 (2.59)	0.25 (2.67)
Median	0.00	0.00
25th, 75th Percentile	0.00, 1.00	0.00, 0.00
Min, Max	-7.0, 6.0	-7.0, 11.0
Week 52		
n	41	43
Mean (SD)	31.85 (5.62)	32.77 (4.19)
Median	35.00	35.00
25th, 75th Percentile	31.00, 35.00	33.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	18.0, 35.0	20.0, 35.0
Change from baseline to Week 52 ^a		
n	40	40
Mean (SD)	-1.10 (4.81)	-0.65 (3.45)
Median	0.00	0.00
25th, 75th Percentile	-2.00, 0.00	0.00, 0.00
Min, Max	-17.0, 8.0	-11.0, 11.0
Difference in change from baseline (95%CI)		0.45 (-1.42, 2.32)
P-value ^b		0.6324
Hedges'g (95% CI) ^c		0.11 (-0.33, 0.54)

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

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Table 14.2.9.1.4.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Non-White		
WeeFIM : Cognitive Score		
Baseline		
n	20	15
Mean (SD)	32.30 (6.00)	33.73 (2.63)
Median	35.00	35.00
25th, 75th Percentile	32.50, 35.00	33.00, 35.00
Min, Max	11.0, 35.0	27.0, 35.0
Week 26		
n	19	14
Mean (SD)	32.47 (6.46)	34.00 (2.29)
Median	35.00	35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.004.007_qs_sum_ovrtm_weef_cog_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
25th, 75th Percentile	35.00, 35.00	35.00, 35.00
Min, Max	11.0, 35.0	27.0, 35.0
Change from baseline to Week 26 ^a		
n	19	14
Mean (SD)	0.32 (2.11)	0.21 (3.47)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-4.0, 6.0	-8.0, 8.0
Week 52		
n	19	14
Mean (SD)	32.32 (6.23)	34.07 (2.30)
Median	35.00	35.00
25th, 75th Percentile	34.00, 35.00	35.00, 35.00

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.004.007_qs_sum_ovrtm_weef_cog_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.9.1.4.7
Functional Independence Measure For Children (WeeFIM) Over Time by Ethnicity: Cognitive Score for BMN111-301
Analysis Population: Full Analysis Set

Ethnicity Score Visit Result	Placebo (N=61)	15 ug/kg BMN 111 (N=60)
Min, Max	11.0, 35.0	27.0, 35.0
Change from baseline to Week 52 ^a		
n	19	14
Mean (SD)	0.16 (1.68)	0.29 (3.75)
Median	0.00	0.00
25th, 75th Percentile	0.00, 0.00	0.00, 0.00
Min, Max	-3.0, 6.0	-8.0, 8.0
Difference in change from baseline (95%CI)		0.13 (-2.14, 2.39)
P-value ^b		0.9066
Hedges'g (95% CI) ^c		0.05 (-0.65, 0.74)
P-value for interaction term, treatment * [Ethnicity]		0.8407

Max, maximum; Min, minimum; SD, standard deviation.

^a Change from baseline is based on the subjects with available measurements at both time points.

^b Two-sided p-value.

^c An effect size that represents standardized mean difference (SMD). The two-sided confidence interval of the SMD is based on a noncentral t-distribution.

Score range 0-35: A higher score reflects a higher level of independence.

Report: mi897809 20JUN2023 11:40 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.09.001.004.007_qs_sum_ovrtm_weef_cog_eth_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovrtm_hedge_sub_301.sas, Database: N/A

Table 14.2.10.4.1
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
BMN111-301: Total Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3362
Baseline Age Group*Treatment Interaction	0.6611
Baseline Tanner Stage*Treatment Interaction	0.4105
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.9547
Baseline AGV Category*Treatment Interaction	0.1622
Ethnicity*Treatment Interaction	0.2332
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.001.000_qs_ped_ctot_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.2

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
BMN111-301: Physical Health Summary Score
Analysis Population: Full Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.2162
Baseline Age Group*Treatment Interaction	0.7788
Baseline Tanner Stage*Treatment Interaction	0.5368
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.8381
Baseline AGV Category*Treatment Interaction	0.0233
Ethnicity*Treatment Interaction	0.2166
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.002.000_qs_ped_cphy_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.3

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
BMN111-301: Psychosocial Summary Score
Analysis Population: Full Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5403
Baseline Age Group*Treatment Interaction	0.3436
Baseline Tanner Stage*Treatment Interaction	0.3962
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.9479
Baseline AGV Category*Treatment Interaction	0.5913
Ethnicity*Treatment Interaction	0.3529
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.003.000_qs_ped_cpsy_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.4

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
BMN111-301: Emotional Functioning Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.5631
Baseline Age Group*Treatment Interaction	0.2856
Baseline Tanner Stage*Treatment Interaction	0.2407
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.8178
Baseline AGV Category*Treatment Interaction	0.5487
Ethnicity*Treatment Interaction	0.9478
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.004.000_qs_ped_cemo_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.5
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
 BMN111-301: Social Functioning Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3393
Baseline Age Group*Treatment Interaction	0.5789
Baseline Tanner Stage*Treatment Interaction	0.7962
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.7786
Baseline AGV Category*Treatment Interaction	0.7402
Ethnicity*Treatment Interaction	0.2207
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.005.000_qs_ped_csoc_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.6

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
 BMN111-301: School Functioning Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9850
Baseline Age Group*Treatment Interaction	0.1530
Baseline Tanner Stage*Treatment Interaction	0.4612
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.7158
Baseline AGV Category*Treatment Interaction	0.6850
Ethnicity*Treatment Interaction	0.3779
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.006.000_qs_ped_csch_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.7
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
BMN111-301: Total Score
Analysis Population: Full Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.4305
Baseline Age Group*Treatment Interaction	0.6854
Baseline Tanner Stage*Treatment Interaction	0.8133
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.6892
Baseline AGV Category*Treatment Interaction	0.4704
Ethnicity*Treatment Interaction	0.6795
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.007.000_qs_ped_stot_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.8
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
 BMN111-301: Physical Health Summary Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.4261
Baseline Age Group*Treatment Interaction	0.8326
Baseline Tanner Stage*Treatment Interaction	0.6316
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.1929
Baseline AGV Category*Treatment Interaction	0.9727
Ethnicity*Treatment Interaction	0.4596
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.008.000_qs_ped_sphy_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.9
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
BMN111-301: Psychosocial Summary Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.4834
Baseline Age Group*Treatment Interaction	0.6672
Baseline Tanner Stage*Treatment Interaction	0.5822
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.8899
Baseline AGV Category*Treatment Interaction	0.2659
Ethnicity*Treatment Interaction	0.8644
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.009.000_qs_ped_spsy_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.10
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
 BMN111-301: Emotional Functioning Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9978
Baseline Age Group*Treatment Interaction	0.2166
Baseline Tanner Stage*Treatment Interaction	0.3834
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.4894
Baseline AGV Category*Treatment Interaction	0.1780
Ethnicity*Treatment Interaction	0.8667
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.010.000_qs_ped_semo_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.11
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
 BMN111-301: Social Functioning Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3297
Baseline Age Group*Treatment Interaction	0.2272
Baseline Tanner Stage*Treatment Interaction	0.7748
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.9416
Baseline AGV Category*Treatment Interaction	0.2697
Ethnicity*Treatment Interaction	0.7230
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.011.000_qs_ped_ssoc_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.4.12
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Pediatric Quality of Life Inventory (PedsQL) at Week 52 for
 BMN111-301: School Functioning Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.5688
Baseline Age Group*Treatment Interaction	0.4129
Baseline Tanner Stage*Treatment Interaction	0.8219
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.4867
Baseline AGV Category*Treatment Interaction	0.8806
Ethnicity*Treatment Interaction	0.8318
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.004.012.000_qs_ped_ssch_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.1

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Total Score
Analysis Population: Full Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.5032
Baseline Age Group*Treatment Interaction	0.1846
Baseline Tanner Stage*Treatment Interaction	0.5562
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.1494
Baseline AGV Category*Treatment Interaction	0.8121
Ethnicity*Treatment Interaction	0.9011
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.001.000_qs_qol_ctot_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.2

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Physical Health Summary Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.6110
Baseline Age Group*Treatment Interaction	0.3426
Baseline Tanner Stage*Treatment Interaction	0.5172
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.1932
Baseline AGV Category*Treatment Interaction	0.5464
Ethnicity*Treatment Interaction	0.8664
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.002.000_qs_qol_cphy_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.3

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
 BMN111-301: Social Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7862
Baseline Age Group*Treatment Interaction	0.1645
Baseline Tanner Stage*Treatment Interaction	0.5947
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.1133
Baseline AGV Category*Treatment Interaction	0.5766
Ethnicity*Treatment Interaction	0.9642
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.003.000_qs_qol_csoc_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.4

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Emotional Functioning Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3156
Baseline Age Group*Treatment Interaction	0.1388
Baseline Tanner Stage*Treatment Interaction	0.0832
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.2433
Baseline AGV Category*Treatment Interaction	0.2879
Ethnicity*Treatment Interaction	0.5725
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.004.000_qs_qol_cemo_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.5

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Coping Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.4585
Baseline Age Group*Treatment Interaction	0.3407
Baseline Tanner Stage*Treatment Interaction	0.0572
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.9526
Baseline AGV Category*Treatment Interaction	0.4528
Ethnicity*Treatment Interaction	0.5011
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.005.000_qs_qol_ccop_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.6

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
 BMN111-301: Beliefs Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7811
Baseline Age Group*Treatment Interaction	0.3409
Baseline Tanner Stage*Treatment Interaction	0.5807
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.0886
Baseline AGV Category*Treatment Interaction	0.7004
Ethnicity*Treatment Interaction	0.5521
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.006.000_qs_qol_cbel_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.7

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Future Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.6434
Baseline Age Group*Treatment Interaction	0.5462
Baseline Tanner Stage*Treatment Interaction	0.8698
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.7188
Baseline AGV Category*Treatment Interaction	0.7593
Ethnicity*Treatment Interaction	0.8178
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.007.000_qs_qol_cfut_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.8

Subgroup*Treatment Interaction P-values from Analysis of Covariance of Caregiver-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Effects Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7307
Baseline Age Group*Treatment Interaction	0.5550
Baseline Tanner Stage*Treatment Interaction	0.7418
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.7044
Baseline AGV Category*Treatment Interaction	0.9311
Ethnicity*Treatment Interaction	0.0981
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.008.000_qs_qol_ceff_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.9
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Total Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9090
Baseline Age Group*Treatment Interaction	0.4014
Baseline Tanner Stage*Treatment Interaction	0.1299
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.3937
Baseline AGV Category*Treatment Interaction	0.7461
Ethnicity*Treatment Interaction	0.1643
Region*Treatment Interaction	NA

NA, not applicable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 27JUL2023 08:34 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.009.000_qs_qol_stot_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.10
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Physical Health Summary Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7639
Baseline Age Group*Treatment Interaction	0.2402
Baseline Tanner Stage*Treatment Interaction	0.2207
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.3535
Baseline AGV Category*Treatment Interaction	0.8957
Ethnicity*Treatment Interaction	0.1438
Region*Treatment Interaction	NA

NA, not applicable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 27JUL2023 08:34 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.010.000_qs_qol_sphy_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.11
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Social Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7545
Baseline Age Group*Treatment Interaction	0.9080
Baseline Tanner Stage*Treatment Interaction	0.1368
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.6090
Baseline AGV Category*Treatment Interaction	0.4433
Ethnicity*Treatment Interaction	0.2262
Region*Treatment Interaction	NA

NA, not applicable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 27JUL2023 08:34 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.011.000_qs_qol_ssoc_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.12
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Emotional Functioning Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.6254
Baseline Age Group*Treatment Interaction	0.3404
Baseline Tanner Stage*Treatment Interaction	0.4936
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.5179
Baseline AGV Category*Treatment Interaction	0.5438
Ethnicity*Treatment Interaction	0.2805
Region*Treatment Interaction	NA

NA, not applicable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 27JUL2023 08:34 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.012.000_qs_qol_semo_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.13
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Coping Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3259
Baseline Age Group*Treatment Interaction	0.4987
Baseline Tanner Stage*Treatment Interaction	0.3125
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.5352
Baseline AGV Category*Treatment Interaction	0.5408
Ethnicity*Treatment Interaction	0.6679
Region*Treatment Interaction	NA

NA, not applicable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 27JUL2023 08:34 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.013.000_qs_qol_scop_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.5.14
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Self-Reported Quality of Life in Short Statured Youth (QoLISSY) at Week 52 for
BMN111-301: Beliefs Score
Analysis Population: Full Analysis Set

<u>Interaction</u>	<u>P-value</u>
Sex*Treatment Interaction	0.3819
Baseline Age Group*Treatment Interaction	0.8813
Baseline Tanner Stage*Treatment Interaction	0.2980
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.9210
Baseline AGV Category*Treatment Interaction	0.8801
Ethnicity*Treatment Interaction	0.8245
Region*Treatment Interaction	NA

NA, not applicable

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 27JUL2023 08:34 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.005.014.000_qs_qol_sbel_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.6.1
Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure For Children (WeeFIM) at Week 52 for
BMN111-301: Total Score
Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.3216
Baseline Age Group*Treatment Interaction	0.6255
Baseline Tanner Stage*Treatment Interaction	0.7341
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.7883
Baseline AGV Category*Treatment Interaction	0.2602
Ethnicity*Treatment Interaction	0.9244
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.006.001.000_qs_weef_tot_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.6.2
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure For Children (WeeFIM) at Week 52 for
 BMN111-301: Self-Care Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.4073
Baseline Age Group*Treatment Interaction	0.5233
Baseline Tanner Stage*Treatment Interaction	0.7582
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.5243
Baseline AGV Category*Treatment Interaction	0.5513
Ethnicity*Treatment Interaction	0.7024
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.006.002.000_qs_weef_sel_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.6.3
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure For Children (WeeFIM) at Week 52 for
 BMN111-301: Mobility Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.0918
Baseline Age Group*Treatment Interaction	0.6300
Baseline Tanner Stage*Treatment Interaction	0.2727
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.3384
Baseline AGV Category*Treatment Interaction	0.8618
Ethnicity*Treatment Interaction	0.5676
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.006.003.000_qs_weef_mob_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.2.10.6.4
 Subgroup*Treatment Interaction P-values from Analysis of Covariance of Functional Independence Measure For Children (WeeFIM) at Week 52 for
 BMN111-301: Cognitive Score
 Analysis Population: Full Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.8068
Baseline Age Group*Treatment Interaction	0.3606
Baseline Tanner Stage*Treatment Interaction	0.7878
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.6830
Baseline AGV Category*Treatment Interaction	0.0849
Ethnicity*Treatment Interaction	0.8407
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 20JUN2023 11:35 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.02.10.006.004.000_qs_weef_cog_int_pval_sub_301_fas.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_qs_sum_ovr_int_pval_sub_301.sas, Database: N/A

Table 14.3.2.2
 Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event: Injection Site Swelling for BMN111-301
 Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7085
Baseline Age Group*Treatment Interaction	NA
Baseline Tanner Stage*Treatment Interaction	0.3269
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	NA
Baseline AGV Category*Treatment Interaction	0.3168
Race*Treatment Interaction	0.1330
Region*Treatment Interaction	NA

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 19JUN2023 07:45 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.002.000.000_ae_iss_int_pval_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_301.sas, Database: N/A

Table 14.3.2.3
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Event of Interest: Injection site reactions (ISR) for BMN111-301
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.2648
Baseline Age Group*Treatment Interaction	0.3731
Baseline Tanner Stage*Treatment Interaction	0.7583
Strata*Treatment Interaction	0.3376
Baseline Height Z-Score Category*Treatment Interaction	0.6700
Baseline AGV Category*Treatment Interaction	0.4026
Race*Treatment Interaction	0.4410
Region*Treatment Interaction	0.3750

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 19JUN2023 07:45 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.003.000.000_ae_eoisr_int_pval_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_301.sas, Database: N/A

Table 14.3.2.4
Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Event of Interest: Hypersensitivity (SMQ) for BMN111-301
Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.7049
Baseline Age Group*Treatment Interaction	0.3158
Baseline Tanner Stage*Treatment Interaction	NA
Strata*Treatment Interaction	NA
Baseline Height Z-Score Category*Treatment Interaction	0.5096
Baseline AGV Category*Treatment Interaction	0.4410
Race*Treatment Interaction	0.5525
Region*Treatment Interaction	0.7261

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

Report: mi897809 19JUN2023 07:45 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.004.000.000_ae_eoihys_int_pval_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_301.sas, Database: N/A

Table 14.3.2.5
 Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing any Adverse Event for BMN111-301
 Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.9642
Baseline Age Group*Treatment Interaction	0.3680
Baseline Tanner Stage*Treatment Interaction	0.2220
Strata*Treatment Interaction	0.3687
Baseline Height Z-Score Category*Treatment Interaction	0.8377
Baseline AGV Category*Treatment Interaction	0.3680
Race*Treatment Interaction	0.2012
Region*Treatment Interaction	0.5725

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

Pvalues are based on relative risk estimates.

Report: mi897809 19JUN2023 07:45 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.02.005.000.000_ae_aae_int_pval_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_301.sas, Database: N/A

Table 14.3.1.2.1
Selected Adverse Events including Treatment Group Comparisons by Sex: Male for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=33)	15 ug/kg BMN 111 (N=31)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	32 (97.0)	30 (96.8)	1.00 (0.91; 1.09) 0.9642	0.94 (0.06; 15.67) 0.9642	-0.0020 (-0.09; 0.08) 0.9642
Injection site reaction	20 (60.6)	23 (74.2)	1.22 (0.87; 1.73) 0.2500	1.87 (0.64; 5.42) 0.2500	0.1359 (-0.09; 0.36) 0.2407
Injection site swelling	4 (12.1)	13 (41.9)	3.46 (1.26; 9.48) 0.0158	5.24 (1.48; 18.56) 0.0104	0.2981 (0.09; 0.50) 0.0046

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 03:52 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.002.001.000_ae_soc_pt_ge10sb_male_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.2.2
Selected Adverse Events including Treatment Group Comparisons by Sex: Female for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=28)	15 ug/kg BMN 111 (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	28 (100.0)	29 (100.0)	NA	NA	NA
Injection site reaction	9 (32.1)	21 (72.4)	2.25 (1.26; 4.04) 0.0063	5.54 (1.78; 17.27) 0.0032	0.4027 (0.17; 0.64) 0.0009
Injection site swelling	2 (7.1)	10 (34.5)	4.83 (1.16; 20.10) 0.0305	6.84 (1.34; 34.90) 0.0207	0.2734 (0.08; 0.47) 0.0067

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.3.1
Selected Adverse Events including Treatment Group Comparisons by Age at Baseline: >=5 to <8 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=24)	15 ug/kg BMN 111 (N=31)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	24 (100.0)	30 (96.8)	0.97 (0.83; 1.15)	0 NA	-0.0323 (-0.17; 0.11)
Injection site reaction	11 (45.8)	26 (83.9)	1.83 (1.15; 2.90)	6.15 (1.76; 21.43)	0.3804 (0.14; 0.62)
Injection site swelling	3 (12.5)	17 (54.8)	4.39 (1.45; 13.25)	8.50 (2.09; 34.52)	0.4234 (0.20; 0.64)
			0.0103	0.0044	0.0017
			0.0088	0.0028	0.0002

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.3.2
Selected Adverse Events including Treatment Group Comparisons by Age at Baseline: >=8 to <11 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=24)	15 ug/kg BMN 111 (N=17)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	24 (100.0)	17 (100.0)	NA	NA	NA
Injection site reaction	13 (54.2)	11 (64.7)	1.19 (0.72; 1.99) 0.4933	1.55 (0.43; 5.57) 0.5008	0.1054 (-0.20; 0.41) 0.4943
Injection site swelling	2 (8.3)	6 (35.3)	4.24 (0.97; 18.51) 0.0551	6.00 (1.04; 34.75) 0.0456	0.2696 (0.02; 0.52) 0.0365

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.3.3
Selected Adverse Events including Treatment Group Comparisons by Age at Baseline: >=11 to <15 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	12 (92.3)	12 (100.0)	1.08 (0.81; 1.56)	NA NA	0.0769 (-0.19; 0.38)
Injection site reaction	5 (38.5)	7 (58.3)	1.52 (0.66; 3.50)	2.24 (0.45; 11.11)	0.1987 (-0.19; 0.58)
Injection site swelling	1 (7.7)	0	0 (0.00; 15.31)	0 (0.00; 20.58)	-0.0769 (-0.38; 0.19)
			NA	NA	NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.4.1
Selected Adverse Events including Treatment Group Comparisons by Baseline Tanner Stage: Tanner Stage I for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=48)	15 ug/kg BMN 111 (N=48)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	48 (100.0)	47 (97.9)	0.98 (0.89; 1.07) NA	0 NA	-0.0208 (-0.11; 0.06) NA
Injection site reaction	26 (54.2)	39 (81.3)	1.50 (1.12; 2.01) 0.0068	3.67 (1.46; 9.21) 0.0057	0.2708 (0.09; 0.45) 0.0030
Injection site swelling	5 (10.4)	22 (45.8)	4.40 (1.82; 10.66) 0.0010	7.28 (2.46; 21.56) 0.0003	0.3542 (0.19; 0.52) 0.0000

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.4.2
Selected Adverse Events including Treatment Group Comparisons by Baseline Tanner Stage: Tanner Stage > I for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	12 (92.3)	12 (100.0)	1.08 (0.81; 1.56)	NA NA	0.0769 (-0.19; 0.38)
Injection site reaction	3 (23.1)	5 (41.7)	1.81 (0.55; 5.98)	2.38 (0.42; 13.39)	0.1859 (-0.18; 0.55)
Injection site swelling	1 (7.7)	1 (8.3)	0.3334 1.08 (0.08; 15.46)	0.3248 1.09 (0.06; 19.63)	0.3127 0.0064 (-0.21; 0.22)
			0.9529	0.9529	0.9530

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.5.1
Selected Adverse Events including Treatment Group Comparisons by Baseline Height Z-score Category: <=-6 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=10)	15 ug/kg BMN 111 (N=15)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	10 (100.0)	15 (100.0)	NA	NA	NA
Injection site reaction	3 (30.0)	8 (53.3)	1.78 (0.62; 5.12) 0.2867	2.67 (0.49; 14.46) 0.2555	0.2333 (-0.15; 0.61) 0.2288
Injection site swelling	1 (10.0)	3 (20.0)	2.00 (0.24; 16.61) 0.5210	2.25 (0.20; 25.37) 0.5118	0.1000 (-0.17; 0.37) 0.4758

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.5.2
Selected Adverse Events including Treatment Group Comparisons by Baseline Height Z-score Category: >-6 to <=-5 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=24)	15 ug/kg BMN 111 (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	23 (95.8)	17 (94.4)	0.99 (0.86; 1.13) 0.8377	0.74 (0.04; 12.67) 0.8349	-0.0139 (-0.15; 0.12) 0.8374
Injection site reaction	10 (41.7)	15 (83.3)	2.00 (1.19; 3.35) 0.0085	7.00 (1.59; 30.80) 0.0100	0.4167 (0.15; 0.68) 0.0018
Injection site swelling	0	10 (55.6)	NA NA NA	NA NA NA	0.5556 (0.31; 0.78) NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.5.3
Selected Adverse Events including Treatment Group Comparisons by Baseline Height Z-score Category: >-5 to <=-4 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=19)	15 ug/kg BMN 111 (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	19 (100.0)	22 (100.0)	NA	NA	NA
Injection site reaction	11 (57.9)	18 (81.8)	1.41 (0.92; 2.17) 0.1158	3.27 (0.79; 13.48) 0.1006	0.2392 (-0.04; 0.51) 0.0874
Injection site swelling	3 (15.8)	10 (45.5)	2.88 (0.93; 8.95) 0.0678	4.44 (1.00; 19.75) 0.0500	0.2967 (0.03; 0.56) 0.0282

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.5.4
Selected Adverse Events including Treatment Group Comparisons by Baseline Height Z-score Category: >-4 for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=8)	15 ug/kg BMN 111 (N=5)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	8 (100.0)	5 (100.0)	NA	NA	NA
Injection site reaction	5 (62.5)	3 (60.0)	0.96 (0.39; 2.35) 0.9287	0.90 (0.09; 8.90) 0.9282	-0.0250 (-0.57; 0.52) 0.9284
Injection site swelling	2 (25.0)	0	0 (0.00; 4.15) NA	0 (0.00; 5.56) NA	-0.2500 (-0.65; 0.30) NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.6.1
Selected Adverse Events including Treatment Group Comparisons by Baseline AGV Category: <=3.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=19)	15 ug/kg BMN 111 (N=19)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	19 (100.0)	18 (94.7)	0.95 (0.73; 1.16) NA	0 NA	-0.0526 (-0.26; 0.13) NA
Injection site reaction	8 (42.1)	16 (84.2)	2.00 (1.14; 3.51) 0.0156	7.33 (1.58; 33.97) 0.0109	0.4211 (0.15; 0.70) 0.0028
Injection site swelling	1 (5.3)	9 (47.4)	9.00 (1.26; 64.26) 0.0285	16.20 (1.78; 147.1) 0.0133	0.4211 (0.18; 0.67) 0.0008

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.6.2
Selected Adverse Events including Treatment Group Comparisons by Baseline AGV Category: => 3.5 to <=4.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=18)	15 ug/kg BMN 111 (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	17 (94.4)	14 (100.0)	1.06 (0.80; 1.38) NA	NA NA	0.0556 (-0.18; 0.27) NA
Injection site reaction	8 (44.4)	10 (71.4)	1.61 (0.87; 2.97) 0.1297	3.13 (0.71; 13.81) 0.1329	0.2698 (-0.06; 0.60) 0.1087
Injection site swelling	1 (5.6)	5 (35.7)	6.43 (0.84; 48.96) 0.0724	9.44 (0.95; 93.64) 0.0551	0.3016 (0.03; 0.57) 0.0300

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.6.3
Selected Adverse Events including Treatment Group Comparisons by Baseline AGV Category: >4.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=24)	15 ug/kg BMN 111 (N=27)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	24 (100.0)	27 (100.0)	NA	NA	NA
Injection site reaction	13 (54.2)	18 (66.7)	1.23 (0.78; 1.94) 0.3706	1.69 (0.54; 5.26) 0.3630	0.1250 (-0.14; 0.39) 0.3591
Injection site swelling	4 (16.7)	9 (33.3)	2.00 (0.71; 5.67) 0.1921	2.50 (0.66; 9.54) 0.1798	0.1667 (-0.07; 0.40) 0.1592

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.7.1
Selected Adverse Events including Treatment Group Comparisons by Ethnicity: White for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=41)	15 ug/kg BMN 111 (N=45)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	40 (97.6)	45 (100.0)	1.03 (0.94; 1.15)	NA NA	0.0244 (-0.06; 0.13)
Injection site reaction	21 (51.2)	37 (82.2)	1.61 (1.16; 2.23)	4.40 (1.65; 11.73)	0.3100 (0.12; 0.50)
Injection site swelling	2 (4.9)	17 (37.8)	7.74 (1.90; 31.49)	11.84 (2.53; 55.42)	0.0013 (0.17; 0.49)
			0.0047	0.0030	0.0017
			0.0042	0.0017	0.0000

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.7.2
Selected Adverse Events including Treatment Group Comparisons by Ethnicity: Non-White for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=20)	15 ug/kg BMN 111 (N=15)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	20 (100.0)	14 (93.3)	0.93 (0.68; 1.12) NA	0 NA	-0.0667 (-0.32; 0.12) NA
Injection site reaction	8 (40.0)	7 (46.7)	1.17 (0.54; 2.50) 0.6918	1.31 (0.34; 5.08) 0.6935	0.0667 (-0.26; 0.40) 0.6934
Injection site swelling	4 (20.0)	6 (40.0)	2.00 (0.68; 5.85) 0.2057	2.67 (0.59; 12.02) 0.2017	0.2000 (-0.10; 0.50) 0.1967

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.8.1
Selected Adverse Events including Treatment Group Comparisons by Region: Japan for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=4)	15 ug/kg BMN 111 (N=3)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	4 (100.0)	3 (100.0)	NA	NA	NA
Injection site reaction	1 (25.0)	2 (66.7)	2.67 (0.41; 17.42) 0.3056	6.00 (0.22; 162.5) 0.2871	0.4167 (-0.26; 1.10) 0.2309
Injection site swelling	0	1 (33.3)	NA NA NA	NA NA NA	0.3333 (-0.36; 0.91) NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.8.2
Selected Adverse Events including Treatment Group Comparisons by Region: North America for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=26)	15 ug/kg BMN 111 (N=27)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	26 (100.0)	26 (96.3)	0.96 (0.81; 1.11) NA	0 NA	-0.0370 (-0.19; 0.10) NA
Injection site reaction	18 (69.2)	18 (66.7)	0.96 (0.67; 1.39) 0.8415	0.89 (0.28; 2.82) 0.8416	-0.0256 (-0.28; 0.23) 0.8414
Injection site swelling	4 (15.4)	14 (51.9)	3.37 (1.28; 8.91) 0.0143	5.92 (1.61; 21.86) 0.0076	0.3647 (0.13; 0.60) 0.0023

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.8.3
Selected Adverse Events including Treatment Group Comparisons by Region: Europe for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=18)	15 ug/kg BMN 111 (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	17 (94.4)	18 (100.0)	1.06 (0.84; 1.40) NA	NA NA	0.0556 (-0.13; 0.27) NA
Injection site reaction	5 (27.8)	17 (94.4)	3.40 (1.60; 7.22) 0.0015	44.20 (4.59; 425.8) 0.0010	0.6667 (0.43; 0.90) 0.0000
Injection site swelling	2 (11.1)	8 (44.4)	4.00 (0.98; 16.30) 0.0531	6.40 (1.12; 36.44) 0.0365	0.3333 (0.06; 0.60) 0.0162

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Table 14.3.1.8.4
Selected Adverse Events including Treatment Group Comparisons by Region: Rest of World for BMN111-301
Analysis Population: Safety Analysis Set

System Organ Class Preferred Term	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any AE, n (%) ^a	13 (100.0)	12 (100.0)	NA	NA	NA
Injection site reaction	5 (38.5)	7 (58.3)	1.52 (0.66; 3.50) 0.3297	2.24 (0.45; 11.11) 0.3237	0.1987 (-0.19; 0.58) 0.3109

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_soc_pt_per_sub_301.sas, Database: N/A

Table 14.3.1.9.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Sex: Male for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=33)	15 ug/kg BMN 111 (N=31)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	29 (87.9)	26 (83.9)	0.95 (0.78; 1.17) 0.6469	0.72 (0.17; 2.96) 0.6458	-0.0401 (-0.21; 0.13) 0.6455
Hypersensitivity (SMQ Narrow Terms)	4 (12.1)	10 (32.3)	2.66 (0.93; 7.61) 0.0679	3.45 (0.95; 12.52) 0.0594	0.2014 (0.00; 0.40) 0.0470

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.001.000_ae_sbj_eoi_male_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.9.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Sex: Male for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=33)	15 ug/kg BMN 111 (N=31)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.001.000_ae_sbj_eoi_male_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.9.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Sex: Male for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=28)	15 ug/kg BMN 111 (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	21 (75.0)	25 (86.2)	1.15 (0.89; 1.49) 0.2914	2.08 (0.54; 8.11) 0.2897	0.1121 (-0.09; 0.32) 0.2808
Hypersensitivity (SMQ Narrow Terms)	3 (10.7)	6 (20.7)	1.93 (0.53; 6.98) 0.3155	2.17 (0.49; 9.71) 0.3093	0.0998 (-0.09; 0.29) 0.2950

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.002.000_ae_sbj_eoi_female_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.9.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Sex: Male for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=28)	15 ug/kg BMN 111 (N=29)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.009.002.000_ae_sbj_eoi_female_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.10.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Age at Baseline: >=5 to <8 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=31)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	19 (79.2)	27 (87.1)	1.10 (0.86; 1.41) 0.4468	1.78 (0.42; 7.50) 0.4342	0.0793 (-0.12; 0.28) 0.4389
Hypersensitivity (SMQ Narrow Terms)	5 (20.8)	8 (25.8)	1.24 (0.46; 3.31) 0.6692	1.32 (0.37; 4.72) 0.6673	0.0497 (-0.17; 0.27) 0.6633

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.001.000_ae_sbj_eoi_age5to8_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.10.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Age at Baseline: >=5 to <8 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=31)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.001.000_ae_sbj_eoi_age5to8_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.10.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Age at Baseline: ≥ 8 to < 11 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=17)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	20 (83.3)	16 (94.1)	1.13 (0.91; 1.40) 0.2668	3.20 (0.32; 31.53) 0.3190	0.1078 (-0.08; 0.29) 0.2568
Hypersensitivity (SMQ Narrow Terms)	1 (4.2)	3 (17.6)	4.24 (0.48; 37.33) 0.1936	4.93 (0.47; 52.13) 0.1850	0.1348 (-0.06; 0.33) 0.1822

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.002.000_ae_sbj_eoi_age8to11_sub_301_saf.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.10.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Age at Baseline: >=8 to <11 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=17)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.002.000_ae_sbj_eoi_age8to11_sub_301_saf.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.10.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Age at Baseline: >=11 to <15 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	11 (84.6)	8 (66.7)	0.79 (0.50; 1.25) 0.3122	0.36 (0.05; 2.50) 0.3033	-0.1795 (-0.51; 0.15) 0.2880
Hypersensitivity (SMQ Narrow Terms)	1 (7.7)	5 (41.7)	5.42 (0.73; 39.97) 0.0975	8.57 (0.83; 89.04) 0.0720	0.3397 (0.03; 0.65) 0.0341

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.003.000_ae_sbj_eoi_age11to15_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.10.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Age at Baseline: ≥ 11 to < 15 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.010.003.000_ae_sbj_eoi_age11to15_sub_301_saf.pdf+rif

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.11.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Tanner Stage: Tanner Stage: I for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=48)	15 ug/kg BMN 111 (N=48)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	40 (83.3)	42 (87.5)	1.05 (0.89; 1.24) 0.5637	1.40 (0.45; 4.39) 0.5642	0.0417 (-0.10; 0.18) 0.5623
Hypersensitivity (SMQ Narrow Terms)	7 (14.6)	14 (29.2)	2.00 (0.89; 4.52) 0.0953	2.41 (0.87; 6.65) 0.0891	0.1458 (-0.02; 0.31) 0.0791

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.011.001.000_ae_sbj_eoi_bltani_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.11.1
 Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Tanner Stage: Tanner Stage: I for BMN111-301
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=48)	15 ug/kg BMN 111 (N=48)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.011.001.000_ae_sbj_eoi_bltani_sub_301_saf.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.11.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Tanner Stage: Tanner Stage: > I for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	10 (76.9)	9 (75.0)	0.98 (0.63; 1.52) 0.9106	0.90 (0.14; 5.65) 0.9105	-0.0192 (-0.35; 0.32) 0.9105
Hypersensitivity (SMQ Narrow Terms)	0	2 (16.7)	NA NA NA	NA NA NA	0.1667 (-0.10; 0.48) NA

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.011.002.000_ae_sbj_eoi_bltangti_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.11.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Tanner Stage: Tanner Stage: > I for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.011.002.000_ae_sbj_eoi_bltangti_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: ≤ -6 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=10)	15 ug/kg BMN 111 (N=15)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	7 (70.0)	10 (66.7)	0.95 (0.55; 1.64) 0.8597	0.86 (0.15; 4.82) 0.8611	-0.0333 (-0.40; 0.34) 0.8602
Hypersensitivity (SMQ Narrow Terms)	1 (10.0)	5 (33.3)	3.33 (0.45; 24.44) 0.2363	4.50 (0.44; 46.17) 0.2055	0.2333 (-0.07; 0.54) 0.1305

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.001.000_ae_sbj_eoi_blhazle6_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.1
 Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: <= -6 for BMN111-301
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=10)	15 ug/kg BMN 111 (N=15)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.001.000_ae_sbj_eoi_blhazle6_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: > -6 to <= -5 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	21 (87.5)	16 (88.9)	1.02 (0.81; 1.27) 0.8897	1.14 (0.17; 7.67) 0.8907	0.0139 (-0.18; 0.21) 0.8898
Hypersensitivity (SMQ Narrow Terms)	3 (12.5)	5 (27.8)	2.22 (0.61; 8.11) 0.2266	2.69 (0.55; 13.20) 0.2221	0.1528 (-0.09; 0.40) 0.2228

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.002.000_ae_sbj_eoi_blhaz6to5_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: > -6 to <= -5 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.002.000_ae_sbj_eoi_blhaz6to5_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: > -5 to <= -4 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=19)	15 ug/kg BMN 111 (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	16 (84.2)	20 (90.9)	1.08 (0.85; 1.37) 0.5238	1.87 (0.28; 12.61) 0.5180	0.0670 (-0.14; 0.27) 0.5183
Hypersensitivity (SMQ Narrow Terms)	2 (10.5)	2 (9.1)	0.86 (0.13; 5.56) 0.8773	0.85 (0.11; 6.69) 0.8773	-0.0144 (-0.20; 0.17) 0.8778

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.003.000_ae_sbj_eoi_blhaz5to4_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.3
 Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: > -5 to <= -4 for BMN111-301
 Analysis Population: Safety Analysis Set

AE Category	Placebo (N=19)	15 ug/kg BMN 111 (N=22)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.003.000_ae_sbj_eoi_blhaz5to4_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.4
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: > -4 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=8)	15 ug/kg BMN 111 (N=5)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	6 (75.0)	5 (100.0)	1.33 (0.60; 2.86) NA	NA NA NA	0.2500 (-0.30; 0.65) NA
Hypersensitivity (SMQ Narrow Terms)	1 (12.5)	4 (80.0)	6.40 (0.97; 42.15) 0.0536	28.00 (1.35; 580.6) 0.0312	0.6750 (0.26; 1.09) 0.0016

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.004.000_ae_sbj_eoi_blhazgt4_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.12.4
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline Height Z-score: > -4 for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=8)	15 ug/kg BMN 111 (N=5)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.012.004.000_ae_sbj_eoi_blhazgt4_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.13.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline AGV: <=3.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=19)	15 ug/kg BMN 111 (N=19)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	18 (94.7)	17 (89.5)	0.94 (0.78; 1.14) 0.5494	0.47 (0.04; 5.70) 0.5548	-0.0526 (-0.22; 0.12) 0.5455
Hypersensitivity (SMQ Narrow Terms)	1 (5.3)	4 (21.1)	4.00 (0.49; 32.57) 0.1951	4.80 (0.48; 47.68) 0.1805	0.1579 (-0.05; 0.37) 0.1387

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.013.001.000_ae_sbj_eoi_blagvle3_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.13.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline AGV: <=3.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=19)	15 ug/kg BMN 111 (N=19)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.13.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline AGV: >3.5 to <=4.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=18)	15 ug/kg BMN 111 (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	14 (77.8)	13 (92.9)	1.19 (0.90; 1.59) 0.2254	3.71 (0.37; 37.71) 0.2671	0.1508 (-0.08; 0.39) 0.2079
Hypersensitivity (SMQ Narrow Terms)	2 (11.1)	6 (42.9)	3.86 (0.91; 16.28) 0.0661	6.00 (0.98; 36.71) 0.0525	0.3175 (0.02; 0.61) 0.0362

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.013.002.000_ae_sbj_eoi_blagv3to4_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.13.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline AGV: >3.5 to <=4.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=18)	15 ug/kg BMN 111 (N=14)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.13.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline AGV: >4.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=27)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	18 (75.0)	21 (77.8)	1.04 (0.76; 1.41) 0.8162	1.17 (0.32; 4.26) 0.8155	0.0278 (-0.21; 0.26) 0.8158
Hypersensitivity (SMQ Narrow Terms)	4 (16.7)	6 (22.2)	1.33 (0.43; 4.17) 0.6207	1.43 (0.35; 5.83) 0.6189	0.0556 (-0.16; 0.27) 0.6148

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.13.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Baseline AGV: >4.5 cm/year for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=24)	15 ug/kg BMN 111 (N=27)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.14.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Ethnicity: White for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=41)	15 ug/kg BMN 111 (N=45)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	35 (85.4)	41 (91.1)	1.07 (0.91; 1.25) 0.4137	1.76 (0.46; 6.73) 0.4108	0.0575 (-0.08; 0.19) 0.4092
Hypersensitivity (SMQ Narrow Terms)	6 (14.6)	13 (28.9)	1.97 (0.83; 4.71) 0.1254	2.37 (0.81; 6.98) 0.1173	0.1425 (-0.03; 0.31) 0.1023

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.14.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Ethnicity: White for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=41)	15 ug/kg BMN 111 (N=45)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.14.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Ethnicity: Non-white for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=20)	15 ug/kg BMN 111 (N=15)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	15 (75.0)	10 (66.7)	0.89 (0.57; 1.38) 0.5984	0.67 (0.15; 2.92) 0.5901	-0.0833 (-0.39; 0.22) 0.5921
Hypersensitivity (SMQ Narrow Terms)	1 (5.0)	3 (20.0)	4.00 (0.46; 34.75) 0.2088	4.75 (0.44; 51.11) 0.1986	0.1500 (-0.07; 0.37) 0.1890

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.014.002.000_ae_sbj_eoi_ethnw_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.14.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Ethnicity: Non-white for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=20)	15 ug/kg BMN 111 (N=15)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: North America for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=26)	15 ug/kg BMN 111 (N=27)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	25 (96.2)	24 (88.9)	0.92 (0.79; 1.08) 0.3172	0.32 (0.03; 3.29) 0.3381	-0.0726 (-0.21; 0.07) 0.3081
Hypersensitivity (SMQ Narrow Terms)	1 (3.8)	5 (18.5)	4.81 (0.60; 38.48) 0.1383	5.68 (0.62; 52.43) 0.1255	0.1467 (-0.02; 0.31) 0.0797

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.001.000_ae_sbj_eoi_regna_sub_301_saf.pdf+rftf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.1
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: North America for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=26)	15 ug/kg BMN 111 (N=27)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: Europe for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=18)	15 ug/kg BMN 111 (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	15 (83.3)	17 (94.4)	1.13 (0.90; 1.43) 0.2966	3.40 (0.32; 36.27) 0.3110	0.1111 (-0.09; 0.31) 0.2812
Hypersensitivity (SMQ Narrow Terms)	2 (11.1)	6 (33.3)	3.00 (0.70; 12.93) 0.1405	4.00 (0.68; 23.41) 0.1241	0.2222 (-0.04; 0.48) 0.0961

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.002.000_ae_sbj_eoi_regeu_sub_301_saf.pdf+rtrf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.2
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: Europe for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=18)	15 ug/kg BMN 111 (N=18)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.002.000_ae_sbj_eoi_regeu_sub_301_saf.pdf+rft

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: Japan for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=4)	15 ug/kg BMN 111 (N=3)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	1 (25.0)	2 (66.7)	2.67 (0.41; 17.42) 0.3056	6.00 (0.22; 162.5) 0.2871	0.4167 (-0.26; 1.10) 0.2309
Hypersensitivity (SMQ Narrow Terms)	1 (25.0)	1 (33.3)	1.33 (0.13; 13.74) 0.8090	1.50 (0.06; 40.63) 0.8096	0.0833 (-0.60; 0.76) 0.8106

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.003.000_ae_sbj_eoi_regip_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.3
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: Japan for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=4)	15 ug/kg BMN 111 (N=3)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.003.000_ae_sbj_eoi_regip_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.4
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: Rest of World for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
Subjects with any EOI, n (%) ^a					
Injection site reactions	9 (69.2)	8 (66.7)	0.96 (0.56; 1.65) 0.8910	0.89 (0.17; 4.78) 0.8908	-0.0256 (-0.39; 0.34) 0.8908
Hypersensitivity (SMQ Narrow Terms)	3 (23.1)	4 (33.3)	1.44 (0.40; 5.17) 0.5718	1.67 (0.29; 9.71) 0.5699	0.1026 (-0.25; 0.45) 0.5675

AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.004.000_ae_sbj_eoi_regrow_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.1.15.4
Selected Adverse Events of Interest including Treatment Group Comparisons by Region: Rest of World for BMN111-301
Analysis Population: Safety Analysis Set

AE Category	Placebo (N=13)	15 ug/kg BMN 111 (N=12)	RR [95%CI] p-value	OR [95%CI] p-value	RD [95%CI] p-value
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AE, adverse event; EOI, event of interest; CTCAE, common terminology criteria for adverse events; MedDRA, Medical Dictionary for Regulatory Activities; NCI, National Cancer Institute; SAE, serious adverse event; RR, relative risk; OR, odds ratio; RD, risk difference; NA, not applicable or not presented.

AEs with onset or worsening after the initiation of study drug and up to 30 days after study drug discontinuation were included. AEs were coded using MedDRA version [Multiple] and graded for severity using NCI CTCAE version 4.0.

^a Percentages were calculated using the total number of subjects in the safety population (N for each treatment group) as the denominator. Subjects with more than one AE of the same category were counted only once for that category.

Report: mi897809 19JUN2023 02:38 /ace/acedev/bmn111/ach/imisc202107a/output/stat/tab/t_14.03.01.015.004.000_ae_sbj_eoi_regrow_sub_301_saf.pdf+rtf

Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_sbj_eoi_sub_301.sas, Database: N/A

Table 14.3.2.1
 Subgroup*Treatment Interaction P-values from a Relative Risk Model of Experiencing an Adverse Event: Injection Site Reaction for BMN111-301
 Analysis Population: Safety Analysis Set

Interaction	P-value
Sex*Treatment Interaction	0.0776
Baseline Age Group*Treatment Interaction	0.4768
Baseline Tanner Stage*Treatment Interaction	0.7681
Strata*Treatment Interaction	0.2174
Baseline Height Z-Score Category*Treatment Interaction	0.5167
Baseline AGV Category*Treatment Interaction	0.4115
Race*Treatment Interaction	0.4509
Region*Treatment Interaction	0.0236

Each interaction term is implemented in a separate model and is the only interaction term used in that respective model.

P-values are based on relative risk estimates.

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Source: /ace/acedev/bmn111/ach/imisc202107a/progstat/t_ae_int_pval_sub_301.sas, Database: N/A