

Dossier zur Nutzenbewertung gemäß § 35a SGB V

Marstacimab (Hympavzi®)

Pfizer Pharma GmbH als örtlicher Vertreter des
Zulassungsinhabers Pfizer Europe MA EEIG

Modul 4 B – Anhang 4-G

*Routineprophylaxe von Blutungsepisoden bei Patienten
ab einem Alter von 12 Jahren mit einem Körpergewicht
von mindestens 35 kg mit Hämophilie B (angeborener
Faktor-IX-Mangel) mit Faktor-IX-Inhibitoren*

Medizinischer Nutzen und
medizinischer Zusatznutzen,
Patientengruppen mit therapeutisch
bedeutsamem Zusatznutzen

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1 Mortalität

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 3.5. Incidence of Adverse Events resulting in Death.

Safety Set

Table page 1 of 1.

Event	N	Incidences by phase		
		Inhibitor with On-Demand at OP n (%)	Marstacimab Prophylaxis	Marstacimab Prophylaxis
			During ATP 6 months n (%)	During ATP 12 months n (%)
Adverse Events Resulting In Death				
Overall				
Safety Set	48	0 (0.0%)	0 (0.0%)	0 (0.0%)
Disease type				
Hemophilia A	40	0 (0.0%)	0 (0.0%)	0 (0.0%)
Hemophilia B	8	0 (0.0%)	0 (0.0%)	0 (0.0%)
Age group				
Adults (Ages 18 to < 75)	36	0 (0.0%)	0 (0.0%)	0 (0.0%)
Adolescents (Ages 12 to < 18)	12	0 (0.0%)	0 (0.0%)	0 (0.0%)
Region				
Asia	24	0 (0.0%)	0 (0.0%)	0 (0.0%)
Europe	8	0 (0.0%)	0 (0.0%)	0 (0.0%)
Other	16	0 (0.0%)	0 (0.0%)	0 (0.0%)
White Race				
White	15	0 (0.0%)	0 (0.0%)	0 (0.0%)
Other	33	0 (0.0%)	0 (0.0%)	0 (0.0%)

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

2 Blutungsereignisse

2.1 ABR behandelter Blutungen

2.1.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 2.1. Annualized Bleeding Rate (ABR) of treated bleeding events.

Modified Intent-to-Treat Set

Table page 1 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Overall		
-		
Descriptive Summary		
n	48	48
Mean	19.81	1.46
SD	14.39	2.70
Median	16.42	0.00
Q1, Q3	9.98, 26.47	0.00, 2.03
Min, Max	0.00, 69.10	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	19.78 (16.12, 24.27)	1.39 (0.85, 2.29)
Ratio Estimate (95% CI), p-value [3]		0.070 (0.042, 0.118), <.0001

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 2.1. Annualized Bleeding Rate (ABR) of treated bleeding events
 Modified Intent-to-Treat Set

Table page 2 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Disease type		
Hemophilia A		
Descriptive Summary		
n	40	40
Mean	18.99	1.08
SD	14.96	1.74
Median	16.05	0.00
Q1, Q3	8.98, 23.44	0.00, 2.01
Min, Max	0.00, 69.10	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	18.97 (14.89, 24.17)	1.04 (0.63, 1.72)
Ratio Estimate (95% CI), p-value [3]		0.055 (0.032, 0.094), <.0001
Hemophilia B		
Descriptive Summary		
n	8	8
Mean	23.89	3.40
SD	10.94	5.20
Median	21.07	1.19
Q1, Q3	16.33, 31.93	0.00, 4.96
Min, Max	9.93, 42.52	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	23.93 (17.74, 32.28)	3.20 (1.21, 8.46)
Ratio Estimate (95% CI), p-value [3]		0.134 (0.049, 0.363), 0.0097
Subgroup interaction model		
Interaction p-value [4]		0.2542

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
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 TABLE 2.1. Annualized Bleeding Rate (ABR) of treated bleeding events
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ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Age group		
Adults (Ages 18 to < 75)		
Descriptive Summary		
n	36	36
Mean	17.19	1.68
SD	14.50	2.89
Median	15.76	0.00
Q1, Q3	7.92, 21.14	0.00, 2.54
Min, Max	0.00, 69.10	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	17.16 (13.06, 22.54)	1.60 (0.94, 2.72)
Ratio Estimate (95% CI), p-value [3]		0.093 (0.052, 0.167), <.0001
Adolescents (Ages 12 to < 18)		
Descriptive Summary		
n	12	12
Mean	27.66	0.82
SD	11.20	2.00
Median	26.47	0.00
Q1, Q3	17.30, 36.99	0.00, 0.50
Min, Max	12.97, 45.19	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	27.80 (22.30, 34.65)	0.83 (0.22, 3.11)
Ratio Estimate (95% CI), p-value [3]		0.030 (0.009, 0.101), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.0448

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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Pfizer Inc.: Final
 Marstacimab
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 TABLE 2.1. Annualized Bleeding Rate (ABR) of treated bleeding events
 Modified Intent-to-Treat Set

Table page 4 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Region		
Smallest subgroup N<10 - analysis not performed		
-		
-	-	-

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
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 TABLE 2.1. Annualized Bleeding Rate (ABR) of treated bleeding events
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ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
White Race		
White		
Descriptive Summary		
n	15	15
Mean	26.22	1.68
SD	18.60	4.06
Median	22.71	0.00
Q1, Q3	13.97, 36.32	0.00, 1.01
Min, Max	1.60, 69.10	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	26.22 (18.51, 37.12)	1.57 (0.49, 5.00)
Ratio Estimate (95% CI), p-value [3]		0.060 (0.018, 0.199), 0.0005
Other		
Descriptive Summary		
n	33	33
Mean	16.89	1.37
SD	11.16	1.86
Median	16.05	0.00
Q1, Q3	9.93, 21.87	0.00, 2.03
Min, Max	0.00, 45.19	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	16.86 (13.47, 21.09)	1.32 (0.83, 2.10)
Ratio Estimate (95% CI), p-value [3]		0.078 (0.048, 0.127), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.6377

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

2.1.2 Sensitivitätsanalyse

Table: Supplementary/Sensitivity Analyses of ABR for Treated Bleeds for Inhibitor Cohort with On-Demand at OP -MITT Set(Protocol B7841005_IN_CSR)

Supplementary Analysis Type	Estimated ABR ¹ Ratio: Marstacimab/On Demand	95% CI
Analysis to assess the impact of preventative infusion		
Including bleeding during preventative infusion	0,070	(0,042; 0,117)
Analyses to assess the impact of marstacimab dose increase		
Number of participants with dose increase: 4		
Including data after a dose increase	0,075	(0,046; 0,121)
Imputing the portion after a dose increase using the data before the dose increase	0,075	(0,043; 0,129)
Tipping point analysis ²	Tipping Multiplier: 14	
	0,190	(0,069; 0,525)
Analyses to assess the impact of treatment discontinuation or potentially missing bleeding records		
Number of participants with ATP discontinuation or dosing gap (≥ 35 days) during which time no bleed was reported: 3		
Imputing the portion after treatment discontinuation using the data during treatment and during dosing gap using the data before and after the dosing gap	0,074	(0,046; 0,121)
Tipping point analysis ²	Tipping Multiplier: 196	
	0,202	(0,081; 0,500)
Analysis to assess the impact of carryover effect from the observational phase		
Excluding the first month data after initiation of marstacimab	0,065	(0,037; 0,112)
Analysis to assess the seasonal effect on bleeding		
Include only the portion of ATP that match the calendar time of OP	0,048	(0,026; 0,087)
1. ABR= annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period / 365.25). If a participant did not complete atreatment period, the days on treatment ended at the last dosing date + 6 days. 2. Using ABRs calculated combining bleeds before the event (dose increase or treatment discontinuation) and assuming the bleed rate after the event as multiples of successively larger (> 1) factors of the former. Note: All analyses used the same model used in the primary efficacy analysis.		

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(Data cutoff date : 29APR2025 Database snapshot date : 03JUN2025) Output File: ./nda1_cdisc/B7841005_IN_CSR/adhe_s1002

Table 14.2.1.1.2i Marstacimab is for Pfizer internal use.

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2.2 ABR von Gelenkblutungen

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
Datacut: B7841005 (29APR2025)
TABLE 2.2. Annualized Bleeding Rate (ABR) of joint bleeds.
Modified Intent-to-Treat Set

Table page 1 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Overall		
-		
Descriptive Summary		
n	48	48
Mean	15.17	1.15
SD	13.17	2.65
Median	11.66	0.00
Q1, Q3	7.17, 17.68	0.00, 1.01
Min, Max	0.00, 57.26	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	15.15 (11.87, 19.34)	1.10 (0.59, 2.04)
Ratio Estimate (95% CI), p-value [3]		0.072 (0.038, 0.138), <.0001

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 2.2. Annualized Bleeding Rate (ABR) of joint bleeds
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Table page 2 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Disease type		
Hemophilia A		
Descriptive Summary		
n	40	40
Mean	14.36	0.69
SD	13.50	1.54
Median	10.18	0.00
Q1, Q3	6.09, 16.72	0.00, 0.51
Min, Max	0.00, 57.26	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	14.35 (10.76, 19.14)	0.69 (0.35, 1.36)
Ratio Estimate (95% CI), p-value [3]		0.048 (0.023, 0.101), <.0001
Hemophilia B		
Descriptive Summary		
n	8	8
Mean	19.22	3.40
SD	11.26	5.20
Median	15.33	1.19
Q1, Q3	10.37, 24.95	0.00, 4.96
Min, Max	9.93, 42.52	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	19.22 (13.07, 28.25)	3.22 (1.22, 8.50)
Ratio Estimate (95% CI), p-value [3]		0.167 (0.066, 0.426), 0.0218
Subgroup interaction model		
Interaction p-value [4]		0.1625

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 2.2. Annualized Bleeding Rate (ABR) of joint bleeds
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Table page 3 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Age group		
Adults (Ages 18 to < 75)		
Descriptive Summary		
n	36	36
Mean	13.21	1.25
SD	13.05	2.85
Median	9.95	0.00
Q1, Q3	5.00, 15.64	0.00, 1.02
Min, Max	0.00, 57.26	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	13.19 (9.58, 18.14)	1.20 (0.59, 2.42)
Ratio Estimate (95% CI), p-value [3]		0.091 (0.043, 0.193), <.0001
Adolescents (Ages 12 to < 18)		
Descriptive Summary		
n	12	12
Mean	21.04	0.82
SD	12.23	2.00
Median	18.63	0.00
Q1, Q3	10.16, 31.67	0.00, 0.50
Min, Max	6.48, 42.52	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	21.12 (15.41, 28.94)	0.83 (0.22, 3.10)
Ratio Estimate (95% CI), p-value [3]		0.039 (0.012, 0.133), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.1955

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

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 TABLE 2.2. Annualized Bleeding Rate (ABR) of joint bleeds
 Modified Intent-to-Treat Set

Table page 4 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Region		
Smallest subgroup N<10 - analysis not performed		
-		
-	-	-

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.2. Annualized Bleeding Rate (ABR) of joint bleeds
 Modified Intent-to-Treat Set

Table page 5 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
White Race		
White		
Descriptive Summary		
n	15	15
Mean	22.36	1.61
SD	17.46	4.08
Median	15.38	0.00
Q1, Q3	9.94, 34.31	0.00, 1.00
Min, Max	0.00, 57.26	0.00, 14.91
Model derived ABR [2]		
Mean estimate (95% CI)	22.35 (15.24, 32.78)	1.52 (0.45, 5.14)
Ratio Estimate (95% CI), p-value [3]		0.068 (0.019, 0.239), 0.0019
Other		
Descriptive Summary		
n	33	33
Mean	11.90	0.93
SD	9.29	1.69
Median	9.93	0.00
Q1, Q3	6.19, 15.22	0.00, 1.02
Min, Max	0.00, 39.21	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	11.88 (9.14, 15.46)	0.93 (0.50, 1.71)
Ratio Estimate (95% CI), p-value [3]		0.078 (0.041, 0.147), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.8187

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

2.3 ABR spontaner Blutungen

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
Datacut: B7841005 (29APR2025)
TABLE 2.3. Annualized Bleeding Rate (ABR) of spontaneous bleeds.
Modified Intent-to-Treat Set

Table page 1 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Overall		
-		
Descriptive Summary		
n	48	48
Mean	15.30	0.89
SD	12.74	1.53
Median	11.49	0.00
Q1, Q3	7.75, 21.67	0.00, 1.68
Min, Max	0.00, 53.89	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	15.27 (12.07, 19.31)	0.87 (0.53, 1.43)
Ratio Estimate (95% CI), p-value [3]		0.057 (0.035, 0.092), <.0001

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.3. Annualized Bleeding Rate (ABR) of spontaneous bleeds
 Modified Intent-to-Treat Set

Table page 2 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Disease type		
Hemophilia A		
Descriptive Summary		
n	40	40
Mean	14.44	0.73
SD	12.95	1.28
Median	10.42	0.00
Q1, Q3	5.05, 21.67	0.00, 1.02
Min, Max	0.00, 53.89	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	14.41 (10.94, 18.98)	0.72 (0.42, 1.23)
Ratio Estimate (95% CI), p-value [3]		0.050 (0.028, 0.088), <.0001
Hemophilia B		
Descriptive Summary		
n	8	8
Mean	19.63	1.66
SD	11.43	2.40
Median	16.72	0.69
Q1, Q3	11.45, 24.95	0.00, 2.51
Min, Max	8.30, 42.52	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	19.55 (13.16, 29.04)	1.59 (0.57, 4.44)
Ratio Estimate (95% CI), p-value [3]		0.081 (0.036, 0.185), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.4074

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.3. Annualized Bleeding Rate (ABR) of spontaneous bleeds
 Modified Intent-to-Treat Set

Table page 3 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Age group		
Adults (Ages 18 to < 75)		
Descriptive Summary		
n	36	36
Mean	13.05	0.94
SD	11.54	1.36
Median	9.98	0.00
Q1, Q3	6.91, 17.87	0.00, 2.01
Min, Max	0.00, 53.89	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	13.02 (9.78, 17.34)	0.91 (0.56, 1.47)
Ratio Estimate (95% CI), p-value [3]		0.070 (0.041, 0.118), <.0001
Adolescents (Ages 12 to < 18)		
Descriptive Summary		
n	12	12
Mean	22.05	0.74
SD	14.26	2.02
Median	21.74	0.00
Q1, Q3	11.89, 32.08	0.00, 0.00
Min, Max	0.00, 43.30	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	22.09 (15.55, 31.39)	0.74 (0.17, 3.26)
Ratio Estimate (95% CI), p-value [3]		0.034 (0.009, 0.124), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.2037

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.3. Annualized Bleeding Rate (ABR) of spontaneous bleeds
 Modified Intent-to-Treat Set

Table page 4 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Region		
Smallest subgroup N<10 - analysis not performed		
-		
-	-	-

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.3. Annualized Bleeding Rate (ABR) of spontaneous bleeds
 Modified Intent-to-Treat Set

Table page 5 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
White Race		
White		
Descriptive Summary		
n	15	15
Mean	17.13	0.82
SD	15.82	1.87
Median	17.39	0.00
Q1, Q3	1.92, 22.71	0.00, 1.01
Min, Max	0.00, 53.89	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	17.07 (10.81, 26.95)	0.79 (0.24, 2.58)
Ratio Estimate (95% CI), p-value [3]		0.046 (0.016, 0.138), <.0001
Other		
Descriptive Summary		
n	33	33
Mean	14.47	0.92
SD	11.25	1.37
Median	10.81	0.00
Q1, Q3	7.85, 18.83	0.00, 2.01
Min, Max	0.00, 43.30	0.00, 6.07
Model derived ABR [2]		
Mean estimate (95% CI)	14.45 (11.11, 18.80)	0.90 (0.54, 1.50)
Ratio Estimate (95% CI), p-value [3]		0.062 (0.037, 0.105), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.6057

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

2.4 ABR von Zielgelenkblutungen

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
Datacut: B7841005 (29APR2025)
TABLE 2.4. Annualized Bleeding Rate (ABR) of target joint bleeds.
Modified Intent-to-Treat Set

Table page 1 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Overall		
-		
Descriptive Summary		
n	48	48
Mean	6.34	0.82
SD	8.51	2.36
Median	2.09	0.00
Q1, Q3	0.00, 10.84	0.00, 0.00
Min, Max	0.00, 42.52	0.00, 13.42
Model derived ABR [2]		
Mean estimate (95% CI)	6.30 (4.32, 9.20)	0.79 (0.36, 1.74)
Ratio Estimate (95% CI), p-value [3]		0.126 (0.062, 0.256), 0.0001

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.4. Annualized Bleeding Rate (ABR) of target joint bleeds
 Modified Intent-to-Treat Set

Table page 2 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Disease type		
Hemophilia A		
Descriptive Summary		
n	40	40
Mean	4.64	0.38
SD	6.43	1.14
Median	0.97	0.00
Q1, Q3	0.00, 8.34	0.00, 0.00
Min, Max	0.00, 24.48	0.00, 5.06
Model derived ABR [2]		
Mean estimate (95% CI)	4.63 (3.03, 7.09)	0.37 (0.15, 0.95)
Ratio Estimate (95% CI), p-value [3]		0.081 (0.032, 0.204), 0.0001
Hemophilia B		
Descriptive Summary		
n	8	8
Mean	14.82	3.04
SD	12.55	4.83
Median	11.00	0.50
Q1, Q3	7.13, 17.90	0.00, 4.96
Min, Max	3.97, 42.52	0.00, 13.42
Model derived ABR [2]		
Mean estimate (95% CI)	14.79 (8.47, 25.81)	2.92 (1.06, 8.01)
Ratio Estimate (95% CI), p-value [3]		0.197 (0.077, 0.506), 0.0532
Subgroup interaction model		
Interaction p-value [4]		0.2761

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.4. Annualized Bleeding Rate (ABR) of target joint bleeds
 Modified Intent-to-Treat Set

Table page 3 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Age group		
Adults (Ages 18 to < 75)		
Descriptive Summary		
n	36	36
Mean	4.81	0.88
SD	6.34	2.49
Median	1.96	0.00
Q1, Q3	0.00, 9.37	0.00, 0.00
Min, Max	0.00, 21.95	0.00, 13.42
Model derived ABR [2]		
Mean estimate (95% CI)	4.78 (3.12, 7.32)	0.85 (0.35, 2.07)
Ratio Estimate (95% CI), p-value [3]		0.177 (0.076, 0.412), 0.0159
Adolescents (Ages 12 to < 18)		
Descriptive Summary		
n	12	12
Mean	10.92	0.66
SD	12.29	1.98
Median	8.21	0.00
Q1, Q3	2.16, 13.43	0.00, 0.00
Min, Max	0.00, 42.52	0.00, 6.87
Model derived ABR [2]		
Mean estimate (95% CI)	10.96 (5.94, 20.21)	0.66 (0.13, 3.38)
Ratio Estimate (95% CI), p-value [3]		0.060 (0.018, 0.199), 0.0005
Subgroup interaction model		
Interaction p-value [4]		0.1011

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.4. Annualized Bleeding Rate (ABR) of target joint bleeds
 Modified Intent-to-Treat Set

Table page 4 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Region		
Smallest subgroup N<10 - analysis not performed		
-		
-	-	-

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.4. Annualized Bleeding Rate (ABR) of target joint bleeds
 Modified Intent-to-Treat Set

Table page 5 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
White Race		
White		
Descriptive Summary		
n	15	15
Mean	10.29	1.42
SD	10.92	3.76
Median	11.35	0.00
Q1, Q3	0.00, 13.75	0.00, 0.00
Min, Max	0.00, 42.52	0.00, 13.42
Model derived ABR [2]		
Mean estimate (95% CI)	10.23 (6.06, 17.27)	1.37 (0.38, 4.92)
Ratio Estimate (95% CI), p-value [3]		0.134 (0.043, 0.415), 0.0224
Other		
Descriptive Summary		
n	33	33
Mean	4.54	0.55
SD	6.60	1.32
Median	2.00	0.00
Q1, Q3	0.00, 6.48	0.00, 0.00
Min, Max	0.00, 24.48	0.00, 5.06
Model derived ABR [2]		
Mean estimate (95% CI)	4.53 (2.77, 7.41)	0.55 (0.24, 1.23)
Ratio Estimate (95% CI), p-value [3]		0.120 (0.054, 0.270), 0.0006
Subgroup interaction model		
Interaction p-value [4]		0.8888

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

2.5 ABR von Gesamtblutungen (behandelt und unbehandelt)

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
Datacut: B7841005 (29APR2025)
TABLE 2.5. Annualized Bleeding Rate (ABR) of total bleeds.
Modified Intent-to-Treat Set

Table page 1 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Overall		
-		
Descriptive Summary		
n	48	48
Mean	27.40	4.48
SD	18.62	8.06
Median	21.91	2.02
Q1, Q3	15.88, 37.28	0.00, 4.79
Min, Max	0.00, 83.83	0.00, 38.66
Model derived ABR [2]		
Mean estimate (95% CI)	27.29 (22.54, 33.03)	4.36 (2.65, 7.18)
Ratio Estimate (95% CI), p-value [3]		0.160 (0.102, 0.249), <.0001

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.5. Annualized Bleeding Rate (ABR) of total bleeds
 Modified Intent-to-Treat Set

Table page 2 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Disease type		
Hemophilia A		
Descriptive Summary		
n	40	40
Mean	27.32	4.39
SD	20.06	8.52
Median	20.72	2.00
Q1, Q3	13.75, 37.28	0.00, 4.31
Min, Max	0.00, 83.83	0.00, 38.66
Model derived ABR [2]		
Mean estimate (95% CI)	27.21 (21.72, 34.09)	4.28 (2.38, 7.72)
Ratio Estimate (95% CI), p-value [3]		0.157 (0.094, 0.264), <.0001
Hemophilia B		
Descriptive Summary		
n	8	8
Mean	27.75	4.95
SD	9.27	5.54
Median	27.95	3.56
Q1, Q3	19.56, 33.93	0.50, 7.56
Min, Max	16.60, 42.52	0.00, 16.40
Model derived ABR [2]		
Mean estimate (95% CI)	27.88 (22.47, 34.58)	4.63 (2.26, 9.49)
Ratio Estimate (95% CI), p-value [3]		0.166 (0.079, 0.350), 0.0037
Subgroup interaction model		
Interaction p-value [4]		0.8388

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.5. Annualized Bleeding Rate (ABR) of total bleeds
 Modified Intent-to-Treat Set

Table page 3 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Age group		
Adults (Ages 18 to < 75)		
Descriptive Summary		
n	36	36
Mean	25.36	5.34
SD	19.60	9.02
Median	19.57	2.99
Q1, Q3	14.30, 31.71	0.00, 5.05
Min, Max	0.00, 83.83	0.00, 38.66
Model derived ABR [2]		
Mean estimate (95% CI)	25.23 (19.65, 32.41)	5.21 (3.03, 8.95)
Ratio Estimate (95% CI), p-value [3]		0.206 (0.130, 0.329), 0.0002
Adolescents (Ages 12 to < 18)		
Descriptive Summary		
n	12	12
Mean	33.50	1.92
SD	14.31	3.01
Median	36.50	0.50
Q1, Q3	19.58, 42.45	0.00, 2.03
Min, Max	12.97, 57.67	0.00, 9.13
Model derived ABR [2]		
Mean estimate (95% CI)	33.55 (26.63, 42.28)	1.92 (0.82, 4.47)
Ratio Estimate (95% CI), p-value [3]		0.057 (0.027, 0.119), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.0057

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 2.5. Annualized Bleeding Rate (ABR) of total bleeds
 Modified Intent-to-Treat Set

Table page 4 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Region		
Smallest subgroup N<10 - analysis not performed		
-		
-	-	-

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 2.5. Annualized Bleeding Rate (ABR) of total bleeds
 Modified Intent-to-Treat Set

Table page 5 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
White Race		
White		
Descriptive Summary		
n	15	15
Mean	34.17	5.96
SD	24.14	10.23
Median	29.94	1.01
Q1, Q3	16.23, 42.52	0.00, 8.24
Min, Max	1.60, 83.83	0.00, 38.66
Model derived ABR [2]		
Mean estimate (95% CI)	34.14 (24.13, 48.29)	5.92 (2.54, 13.82)
Ratio Estimate (95% CI), p-value [3]		0.173 (0.084, 0.357), 0.0040
Other		
Descriptive Summary		
n	33	33
Mean	24.32	3.81
SD	14.94	6.93
Median	19.57	2.02
Q1, Q3	15.71, 32.11	0.00, 4.07
Min, Max	0.00, 66.03	0.00, 32.83
Model derived ABR [2]		
Mean estimate (95% CI)	24.18 (19.67, 29.73)	3.62 (2.03, 6.45)
Ratio Estimate (95% CI), p-value [3]		0.150 (0.087, 0.258), <.0001
Subgroup interaction model		
Interaction p-value [4]		0.7716

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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2.6 ABR traumatischer Blutungen

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
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TABLE 2.7. Annualized Bleeding Rate (ABR) of traumatic bleeds.
Modified Intent-to-Treat Set

Table page 1 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Overall		
-		
Descriptive Summary		
n	48	48
Mean	4.50	0.58
SD	8.32	1.84
Median	2.00	0.00
Q1, Q3	0.00, 6.29	0.00, 0.00
Min, Max	0.00, 51.33	0.00, 11.93
Model derived ABR [2]		
Mean estimate (95% CI)	4.49 (2.67, 7.56)	0.53 (0.22, 1.26)
Ratio Estimate (95% CI), p-value [3]		0.118 (0.044, 0.318), 0.0042

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.7. Annualized Bleeding Rate (ABR) of traumatic bleeds
 Modified Intent-to-Treat Set

Table page 2 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Disease type		
Hemophilia A		
Descriptive Summary		
n	40	40
Mean	4.55	0.34
SD	8.98	0.82
Median	1.93	0.00
Q1, Q3	0.00, 6.04	0.00, 0.00
Min, Max	0.00, 51.33	0.00, 3.02
Model derived ABR [2]		
Mean estimate (95% CI)	4.56 (2.49, 8.33)	0.33 (0.15, 0.69)
Ratio Estimate (95% CI), p-value [3]		0.071 (0.027, 0.190), <.0001
Hemophilia B		
Descriptive Summary		
n	8	8
Mean	4.26	1.74
SD	4.03	4.14
Median	4.16	0.00
Q1, Q3	0.00, 7.88	0.00, 1.01
Min, Max	0.00, 9.98	0.00, 11.93
Model derived ABR [2]		
Mean estimate (95% CI)	4.19 (2.25, 7.83)	1.63 (0.35, 7.56)
Ratio Estimate (95% CI), p-value [3]		0.390 (0.095, 1.605), 0.7304
Subgroup interaction model		
Interaction p-value [4]		0.2716

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
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 Inhibitor with On-Demand at OP
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 TABLE 2.7. Annualized Bleeding Rate (ABR) of traumatic bleeds
 Modified Intent-to-Treat Set

Table page 3 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
Age group		
Adults (Ages 18 to < 75)		
Descriptive Summary		
n	36	36
Mean	4.13	0.74
SD	9.09	2.10
Median	0.00	0.00
Q1, Q3	0.00, 5.01	0.00, 0.50
Min, Max	0.00, 51.33	0.00, 11.93
Model derived ABR [2]		
Mean estimate (95% CI)	4.12 (2.02, 8.40)	0.70 (0.29, 1.71)
Ratio Estimate (95% CI), p-value [3]		0.170 (0.056, 0.514), 0.0561
Adolescents (Ages 12 to < 18)		
Descriptive Summary		
n	12	12
Mean	5.61	0.08
SD	5.58	0.29
Median	4.04	0.00
Q1, Q3	0.94, 9.62	0.00, 0.00
Min, Max	0.00, 15.38	0.00, 1.00
Model derived ABR [2]		
Mean estimate (95% CI)	5.63 (3.28, 9.67)	0.08 (0.01, 0.54)
Ratio Estimate (95% CI), p-value [3]		0.015 (0.002, 0.098), 0.0003
Subgroup interaction model		
Interaction p-value [4]		0.0441

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 2.7. Annualized Bleeding Rate (ABR) of traumatic bleeds
 Modified Intent-to-Treat Set

Table page 4 of 5.

	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
ABR [1]		
Region		
Smallest subgroup N<10 - analysis not performed		
-		
-	-	-

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 2.7. Annualized Bleeding Rate (ABR) of traumatic bleeds
 Modified Intent-to-Treat Set

Table page 5 of 5.

ABR [1]	Inhibitor with On-Demand at OP (N=48)	Marstacimab Prophylaxis During ATP (N=48)
White Race		
White		
Descriptive Summary		
n	15	15
Mean	9.09	0.86
SD	12.91	3.07
Median	7.45	0.00
Q1, Q3	0.00, 11.78	0.00, 0.00
Min, Max	0.00, 51.33	0.00, 11.93
Model derived ABR [2]		
Mean estimate (95% CI)	9.10 (4.54, 18.24)	0.79 (0.14, 4.48)
Ratio Estimate (95% CI), p-value [3]		0.087 (0.013, 0.570), 0.0682
Other		
Descriptive Summary		
n	33	33
Mean	2.42	0.45
SD	3.81	0.89
Median	0.00	0.00
Q1, Q3	0.00, 3.77	0.00, 0.00
Min, Max	0.00, 14.61	0.00, 3.02
Model derived ABR [2]		
Mean estimate (95% CI)	2.40 (1.41, 4.08)	0.42 (0.21, 0.85)
Ratio Estimate (95% CI), p-value [3]		0.176 (0.080, 0.387), 0.0093
Subgroup interaction model		
Interaction p-value [4]		0.4468

ATP: Active Treatment Phase; CI: Confidence Interval; Min: Minimum; Max: Maximum; N: Number of patients; OP: Observational Phase; Q1: 25%-Quartile; Q3: 75%-Quartile; SD: Standard Deviation.

[1] ABR = annualized bleeding rate calculated as the number of bleeds requiring treatment/(days on treatment period /365.25). If a participant did not complete a treatment period, the days on treatment ended at the last dosing date + 6 days.

[2] Based on a repeated measure negative binomial regression model via generalized estimating equation approach with log link function, the working correlation was set as unstructured. The model used the number of bleeds as a response variable, treatment (marstacimab prophylactic or on-demand) as a factor, and log time on treatment as an offset variable to account for different duration on treatment.

[3] P-value for the null hypothesis (ratio = 0.5). Superiority of marstacimab prophylaxis is declared when the 95% CI lies below 0.5.

[4] Based on same negative binomial regression model with additional factors for subgroup and subgroup*treatment interaction.

2.7 Anzahl der Patienten ohne Blutungen

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 5. Incidence of No bleeds or target joint analysis.
 Modified Intent-to-Treat Set

Table page 1 of 2.

Event	N	Incidences by phase				ATP 6-months vs. OP 6-months						OR Interacti on p- value [2]
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	N 12m	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI)		OR (95%CI)		RD (95%CI)		
						p-value [1]		p-value [2]		p-value [3]		
Patients without any bleeds												
Overall												
Modified Intent-to-Treat Set	48	1 (2.1%)	26 (54.2%)	45	15 (33.3%)	26.00	(3.80, 177.68)	55.55	(7.62, 404.68)	52.1	(38.0, 66.2)	
							0.0009		<.0001		<.0001	
Disease type												
Hemophilia A	40	1 (2.5%)	21 (52.5%)	38	13 (34.2%)	21.00	(3.10, 142.20)	43.11	(5.91, 314.53)	50.0	(34.5, 65.5)	
							0.0018		0.0002		<.0001	
Hemophilia B	8	0 (0.0%)	5 (62.5%)	7	2 (28.6%)	6.00	(0.72, 49.84)	21.00	(0.72, 609.25)	62.5	(14.3, 110.7)	0.7377
							0.0971		0.0764		0.0111	
Age group												
Adults (Ages 18 to < 75)	36	1 (2.8%)	17 (47.2%)	33	9 (27.3%)	17.00	(2.54, 113.82)	31.32	(4.33, 226.48)	44.4	(28.2, 60.7)	
							0.0035		0.0006		<.0001	
Adolescents (Ages 12 to < 18)	12	0 (0.0%)	9 (75.0%)	12	6 (50.0%)	10.00	(1.56, 64.20)	55.00	(5.10, 593.40)	75.0	(50.5, 99.5)	0.7285
							0.0152		0.0010		<.0001	
Region												
Asia	24	0 (0.0%)	11 (45.8%)	23	4 (17.4%)		-		-		-	
Europe	8	0 (0.0%)	5 (62.5%)	7	4 (57.1%)		-		-		-	
Other	16	1 (6.3%)	10 (62.5%)	15	7 (46.7%)		-		-		-	-
White Race												
White	15	0 (0.0%)	9 (60.0%)	13	5 (38.5%)	10.00	(1.56, 64.20)	28.00	(3.40, 230.57)	60.0	(35.2, 84.8)	
							0.0152		0.0020		<.0001	
Other	33	1 (3.0%)	17 (51.5%)	32	10 (31.3%)	17.00	(2.54, 113.82)	34.00	(4.65, 248.82)	48.5	(31.4, 65.5)	0.8960
							0.0035		0.0005		<.0001	

Bleedings with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. Bleedings with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. Bleedings data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

Patients who did not complete the ATP were conservatively excluded, as subsequent bleeds could not be ruled out and time-adjustment is not feasible in responder analyses. Patients who discontinued after 6 months were included in the 6-month ATP analysis but excluded from the 12-month ATP count.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

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

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

2.8 Anzahl der Patienten ohne Zielgelenke

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 5. Incidence of No bleeds or target joint analysis

Modified Intent-to-Treat Set

Table page 2 of 2.

Event	N	Incidences by phase				ATP 6-months vs. OP 6-months							
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	N 12m	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI)		OR (95%CI)		RD (95%CI)		OR Interacti on p- value [2]	
						p-value [1]		p-value [2]		p-value [3]			
Patients without any target joint													
Overall													
Modified Intent-to-Treat Set	48	27 (56.3%)	47 (97.9%)	45	43 (95.6%)	1.74	(1.36, 2.23)	36.56	(5.11, 261.72)	41.7	(27.7, 55.6)		
							<.0001		0.0003		<.0001		
Disease type													
Hemophilia A	40	24 (60.0%)	40 (100.0%)	38	37 (97.4%)	1.70	(1.28, 2.24)	28.83	(3.32, 250.53)	40.0	(23.3, 56.7)		
							0.0002		0.0023		<.0001		
Hemophilia B	8	3 (37.5%)	7 (87.5%)	7	6 (85.7%)	2.33	(0.99, 5.49)	11.67	(1.34, 101.57)	50.0	(15.4, 84.6)	0.5796	
							0.0522		0.0261		0.0047		
Age group													
Adults (Ages 18 to < 75)	36	23 (63.9%)	35 (97.2%)	33	32 (97.0%)	1.52	(1.20, 1.93)	19.78	(2.81, 139.17)	33.3	(17.9, 48.7)		
							0.0006		0.0027		<.0001		
Adolescents (Ages 12 to < 18)	12	4 (33.3%)	12 (100.0%)	12	11 (91.7%)	3.67	(1.40, 9.62)	33.00	(3.55, 306.47)	66.7	(40.0, 93.3)	0.7426	
							0.0083		0.0021		<.0001		
Region													
Asia	24	11 (45.8%)	24 (100.0%)	23	22 (95.7%)		-		-		-		
Europe	8	3 (37.5%)	8 (100.0%)	7	6 (85.7%)		-		-		-		
Other	16	13 (81.3%)	15 (93.8%)	15	15 (100.0%)		-		-		-		-
White Race													
White	15	7 (46.7%)	14 (93.3%)	13	12 (92.3%)	2.00	(1.18, 3.38)	16.00	(2.10, 121.67)	46.7	(21.4, 71.9)		
							0.0095		0.0074		0.0003		
Other	33	20 (60.6%)	33 (100.0%)	32	31 (96.9%)	1.68	(1.24, 2.29)	23.58	(2.60, 213.53)	39.4	(20.7, 58.1)	0.8009	
							0.0009		0.0049		<.0001		

Bleedings with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. Bleedings with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. Bleedings data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

Patients who did not complete the ATP were conservatively excluded, as subsequent bleeds could not be ruled out and time-adjustment is not feasible in responder analyses. Patients who discontinued after 6 months were included in the 6-month ATP analysis but excluded from the 12-month ATP count.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

3 Gesundheitszustand

3.1 EQ-5D-VAS

3.1.1 Hauptanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.1.8. EQ-5D-VAS score response (≥ 15 points Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Overall								
-	48	10.6 (22.1%)	17.0 (35.4%)	19.3 (40.2%)	1.61 (0.83, 3.09) 0.1563	1.94 (0.79, 4.77) 0.1503	13.3 (-4.5, 31.1) 0.1424	
Disease type								
Hemophilia A	40	9.5 (23.8%)	13.0 (32.5%)	16.3 (40.8%)	1.37 (0.69, 2.71) 0.3649	1.55 (0.61, 3.96) 0.3616	8.8 (-9.9, 27.4) 0.3579	
Hemophilia B	8	1.1 (13.8%)	4.0 (50.0%)	3.0 (37.5%)	3.73 (0.42, 33.49) 0.2394	6.43 (0.34, 122.12) 0.2152	36.3 (-13.6, 86.1) 0.1545	0.3676
Age group								
Adults (Ages 18 to < 75)	36	7.5 (20.8%)	11.0 (30.6%)	13.3 (36.9%)	1.47 (0.64, 3.39) 0.3660	1.68 (0.55, 5.09) 0.3625	9.7 (-11.0, 30.4) 0.3573	
Adolescents (Ages 12 to < 18)	12	3.1 (25.8%)	6.0 (50.0%)	6.0 (50.0%)	1.94 (0.68, 5.52) 0.2126	2.88 (0.59, 14.09) 0.1913	24.2 (-10.5, 58.8) 0.1717	0.5851
Region								
Asia	24	5.0 (20.8%)	8.0 (33.3%)	11.3 (47.1%)	-	-	-	
Europe	8	2.0 (25.0%)	4.0 (50.0%)	3.0 (37.5%)	-	-	-	
Other	16	3.6 (22.5%)	5.0 (31.3%)	5.0 (31.3%)	-	-	-	-
White Race								
White	15	5.0 (33.3%)	8.0 (53.3%)	7.0 (46.7%)	1.60 (0.63, 4.05) 0.3218	2.29 (0.46, 11.44) 0.3143	20.0 (-17.9, 57.9) 0.3006	
Other	33	5.6 (17.0%)	9.0 (27.3%)	12.3 (37.3%)	1.61 (0.64, 4.05) 0.3088	1.84 (0.58, 5.89) 0.3029	10.3 (-9.0, 29.6) 0.2949	0.8312

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visits data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD were performed by imputation, and the results were pooled using Rubin's rules.

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In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

3.1.2 Sensitivitätsanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 4 TABLE 6.1.45. EQ-5D Visual Analog Scale (EQ-VAS) score.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=48			
OP Baseline	n=48 70.1 (23.15) 50.0 72.00 89.5	-	-
OP Day 180	n=44 72.3 (19.82) 60.0 75.00 90.0	n=44 3.5 (25.22) -10.0 0.00 10.5	1.5 (-6.2, 9.3)
ATP Baseline	n=48 71.4 (24.35) 56.5 76.50 90.0	-	-
ATP Day 180	n=44 81.3 (20.33) 70.0 90.00 95.5	n=44 9.9 (19.49) 0.0 7.50 16.5	10.2 (4.2, 16.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	7.5 (-1.9, 17.0)
ATP Day 360	n=47 85.3 (12.77) 77.0 88.00 98.0	n=47 14.1 (23.92) 0.0 5.00 21.0	0.1184 13.3 (4.3, 22.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. EQ5D-VAS measures the participant's self-rated health state on a scale from 0 (worst imaginable health state) to 100 (best imaginable health state).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.45. EQ-5D Visual Analog Scale (EQ-VAS) score
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=40			
OP Baseline	n=40 71.0 (24.16) 50.0 79.00 90.0	-	-
OP Day 180	n=37 74.1 (19.69) 60.0 79.00 90.0	n=37 4.6 (25.55) -9.0 0.00 10.0	2.5 (-7.1, 12.1)
ATP Baseline	n=40 72.5 (23.68) 59.0 79.00 90.0	-	-
ATP Day 180	n=38 80.9 (21.52) 70.0 90.00 96.0	n=38 8.8 (19.67) 0.0 4.50 16.0	9.3 (1.9, 16.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	2.9 (-8.9, 14.7)
ATP Day 360	n=39 85.2 (13.39) 77.0 90.00 98.0	n=39 12.9 (24.04) 0.0 3.00 20.0	0.5971 11.7 (2.1, 21.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. EQ5D-VAS measures the participant's self-rated health state on a scale from 0 (worst imaginable health state) to 100 (best imaginable health state).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.45. EQ-5D Visual Analog Scale (EQ-VAS) score
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=8			
OP Baseline	n=8 65.6 (17.82) 52.0 69.50 77.0	-	-
OP Day 180	n=7 62.7 (19.02) 50.0 50.00 70.0	n=7 -2.4 (24.34) -21.0 -4.00 11.0	-4.4 (-25.3, 16.4)
ATP Baseline	n=8 65.5 (28.46) 40.0 70.00 89.5	-	-
ATP Day 180	n=6 83.7 (10.91) 80.0 82.50 91.0	n=6 16.8 (18.31) -1.0 12.50 37.0	16.5 (-5.9, 38.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	20.0 (-16.8, 56.8)
ATP Day 360	n=8 85.6 (9.93) 78.5 81.50 95.0	n=8 20.1 (23.91) 1.0 7.50 46.0	0.2533 25.3 (-3.2, 53.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

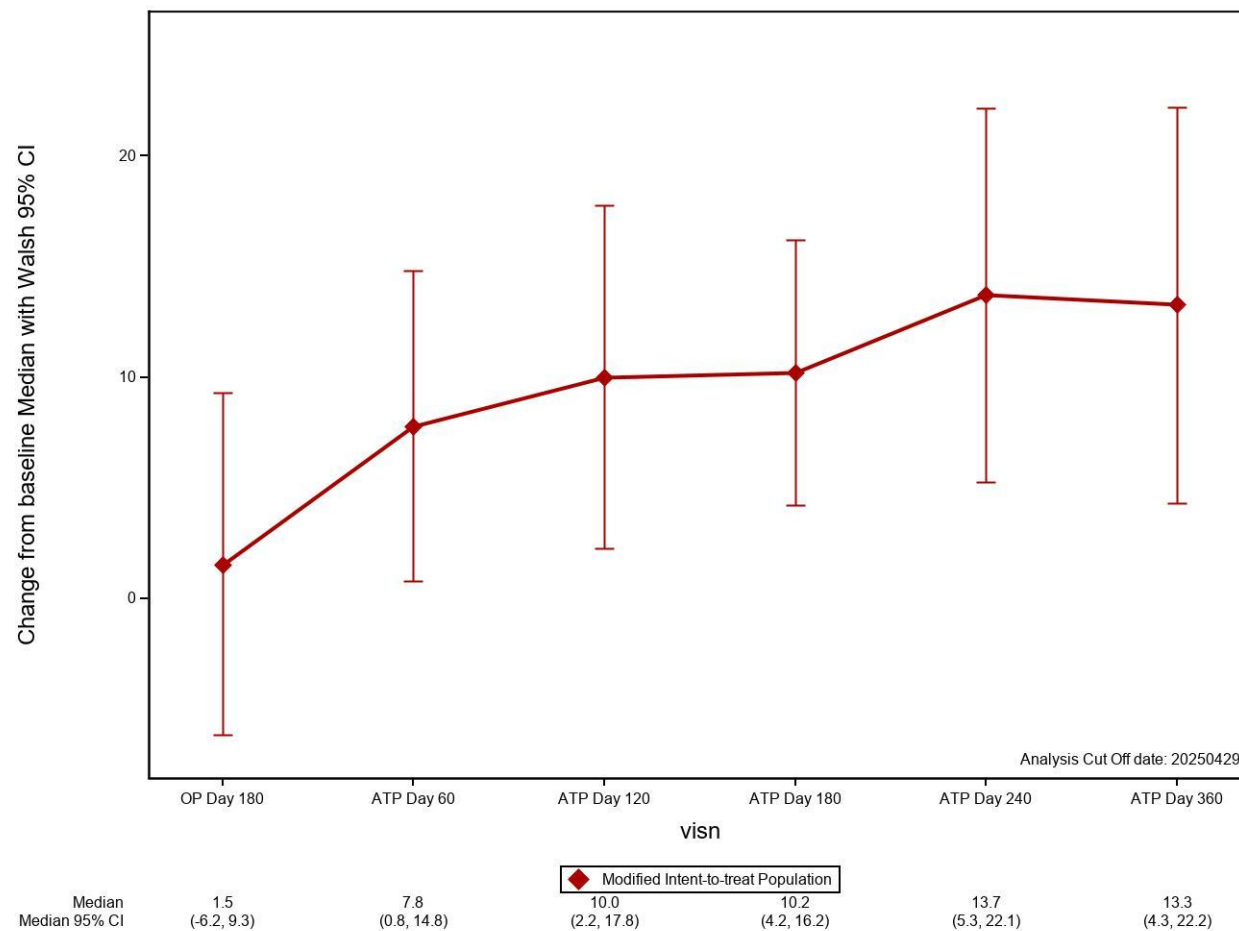
Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

EQ5D-VAS measures the participant's self-rated health state on a scale from 0 (worst imaginable health state) to 100 (best imaginable health state).

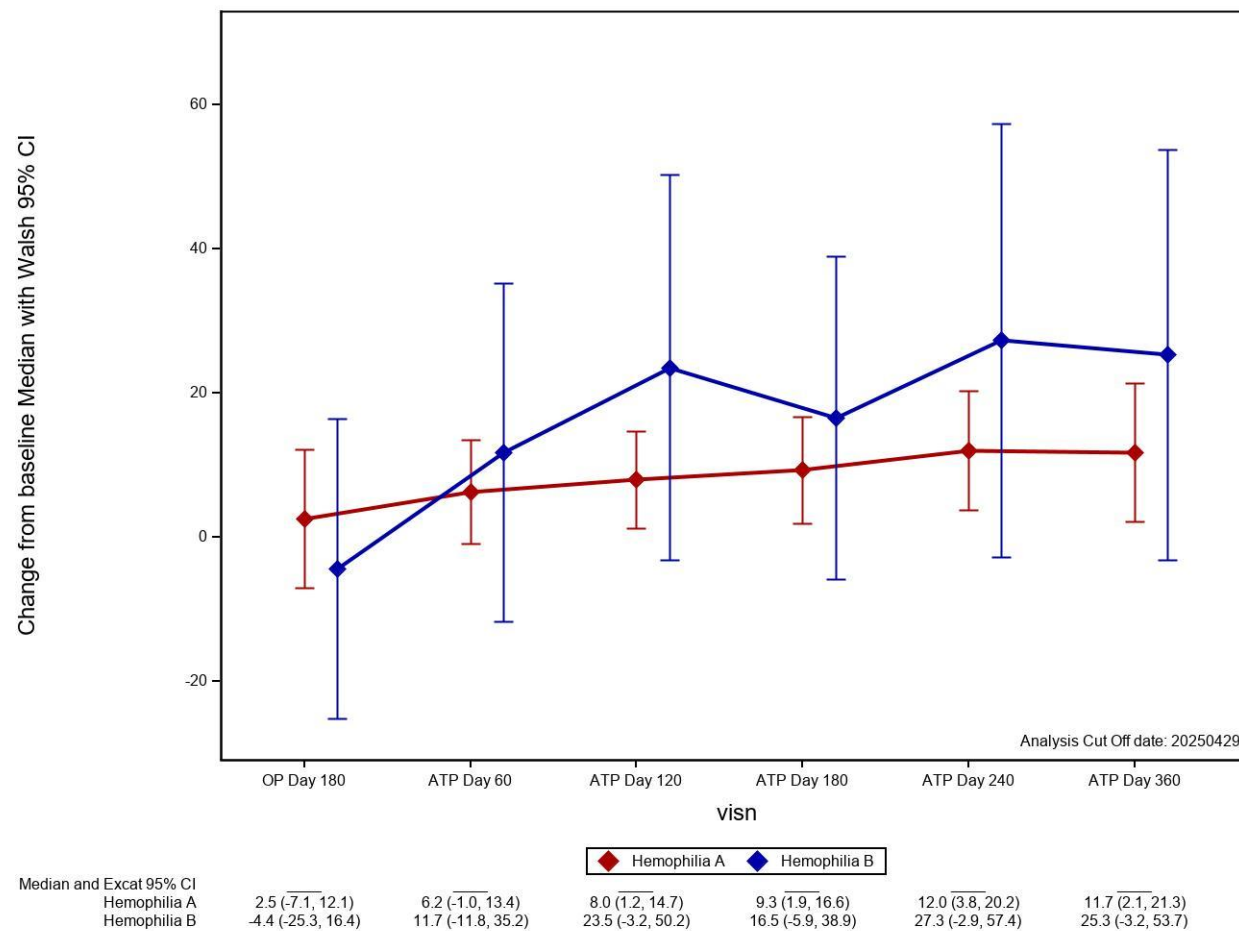
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.45.1: EQ-5D Visual Analog Scale (EQ-VAS) score - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



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FIGURE .6.2.45.2: EQ-5D Visual Analog Scale (EQ-VAS) score - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



4.1 PGIC-H

4.1.1 Hauptanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.1.7. PGIC response (at least Slight Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months					OR Interacti on p- value [2]
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	
Overall										
-	48	26.0 (54.2%)	47.0 (97.9%)	47.0 (97.9%)	1.81 (1.38, 2.38) <.0001		39.76 (5.48, 288.54) 0.0003	43.8 (28.9, 58.6) <.0001		
Disease type										
Hemophilia A	40	23.5 (58.8%)	39.0 (97.5%)	39.0 (97.5%)	1.66 (1.27, 2.17) 0.0002		27.37 (3.81, 196.74) 0.0010	38.8 (23.0, 54.5) <.0001		
Hemophilia B	8	2.5 (31.3%)	8.0 (100.0%)	8.0 (100.0%)	4.95 (0.89, 27.38) 0.0667		32.08 (2.12, 484.92) 0.0124	68.8 (34.1, 103.4) 0.0001	0.9256	
Age group										
Adults (Ages 18 to < 75)	36	18.5 (51.4%)	36.0 (100.0%)	35.0 (97.2%)	2.00 (1.42, 2.83) <.0001		37.00 (5.02, 272.69) 0.0004	48.6 (31.6, 65.6) <.0001		
Adolescents (Ages 12 to < 18)	12	7.5 (62.5%)	11.0 (91.7%)	12.0 (100.0%)	1.47 (0.95, 2.26) 0.0806		6.57 (0.93, 46.59) 0.0595	29.2 (2.0, 56.4) 0.0356	0.2243	
Region										
Asia	24	15.8 (65.8%)	24.0 (100.0%)	24.0 (100.0%)	-		-	-		
Europe	8	3.0 (37.5%)	8.0 (100.0%)	7.0 (87.5%)	-		-	-		
Other	16	7.2 (45.0%)	15.0 (93.8%)	16.0 (100.0%)	-		-	-	-	
White Race										
White	15	5.0 (33.3%)	14.0 (93.3%)	14.0 (93.3%)	2.80 (1.39, 5.65) 0.0041		28.00 (3.40, 230.57) 0.0020	60.0 (35.2, 84.8) <.0001		
Other	33	21.0 (63.6%)	33.0 (100.0%)	33.0 (100.0%)	1.60 (1.19, 2.15) 0.0019		20.76 (2.86, 150.71) 0.0027	36.4 (18.4, 54.3) <.0001	0.8393	

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visis data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD where performed by imputation, and the results were pooled using Rubin's rules.

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

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

4.1.2 Sensitivitätsanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 5 TABLE 6.1.44. Patient Global Impression of Change (PGIC-H) - PGI01-Improvement.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=48			
OP Day 180	n=43 2.9 (1.39) 2.0 3.00 4.0		3.0 (2.5, 3.4)
ATP Baseline	n=1 4.0 (-) 4.0 4.00 4.0	-	-
ATP Day 180	n=45 1.4 (0.81) 1.0 1.00 1.0		NE
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-2.0 (-2.5, -1.5) <.0001
ATP Day 360	n=47 1.4 (0.80) 1.0 1.00 2.0		NE

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. PIGC scores are responses to the question, 'Since the start of the study, my life with hemophilia has:' at OP day 180; And 'Since you began receiving injections as part of this study, life with hemophilia has been:' at all ATP visits with scores: 1 = Greatly improved, 2 = Moderately improved, 3 = Slightly improved, 4 = Not changed, 5 = Slightly worsened, 6 = Moderately worsened, and 7 = Greatly worsened.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.44. Patient Global Impression of Change (PGIC-H) - PGI01-Improvement
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=40			
OP Day 180	n=36 2.8 (1.39) 1.5 3.00 4.0		2.9 (2.2, 3.5)
ATP Baseline	n=1 4.0 (-) 4.0 4.00 4.0	-	-
ATP Day 180	n=38 1.4 (0.86) 1.0 1.00 1.0		1.0 (0.7, 1.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-2.0 (-2.6, -1.4) <.0001
ATP Day 360	n=39 1.4 (0.85) 1.0 1.00 2.0		1.0 (0.7, 1.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. PIGC scores are responses to the question, 'Since the start of the study, my life with hemophilia has:' at OP day 180; And 'Since you began receiving injections as part of this study, life with hemophilia has been:' at all ATP visits with scores: 1 = Greatly improved, 2 = Moderately improved, 3 = Slightly improved, 4 = Not changed, 5 = Slightly worsened, 6 = Moderately worsened, and 7 = Greatly worsened.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.44. Patient Global Impression of Change (PGIC-H) - PGI01-Improvement
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=8			
OP Day 180	n=7 3.6 (1.27) 3.0 4.00 4.0		4.0 (2.9, 5.1)
ATP Day 180	n=7 1.3 (0.49) 1.0 1.00 2.0		1.0 (0.7, 1.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-2.5 (-3.4, -1.6) <.0001
ATP Day 360	n=8 1.4 (0.52) 1.0 1.00 2.0		1.0 (0.7, 1.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

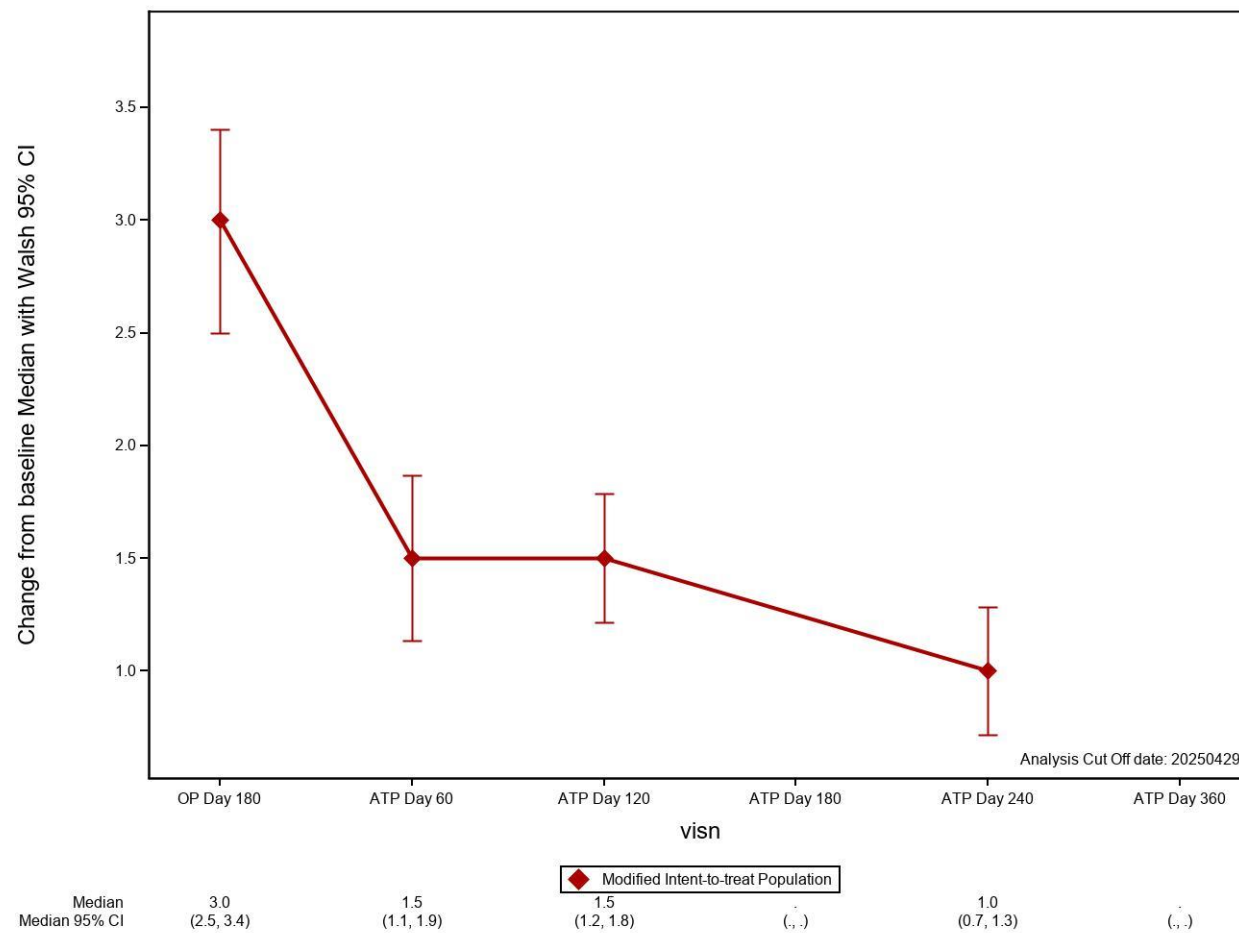
Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption.

Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

PGIC scores are responses to the question, 'Since the start of the study, my life with hemophilia has:' at OP day 180; And 'Since you began receiving injections as part of this study, life with hemophilia has been:' at all ATP visits with scores: 1 = Greatly improved, 2 = Moderately improved, 3 = Slightly improved, 4 = Not changed, 5 = Slightly worsened, 6 = Moderately worsened, and 7 = Greatly worsened.

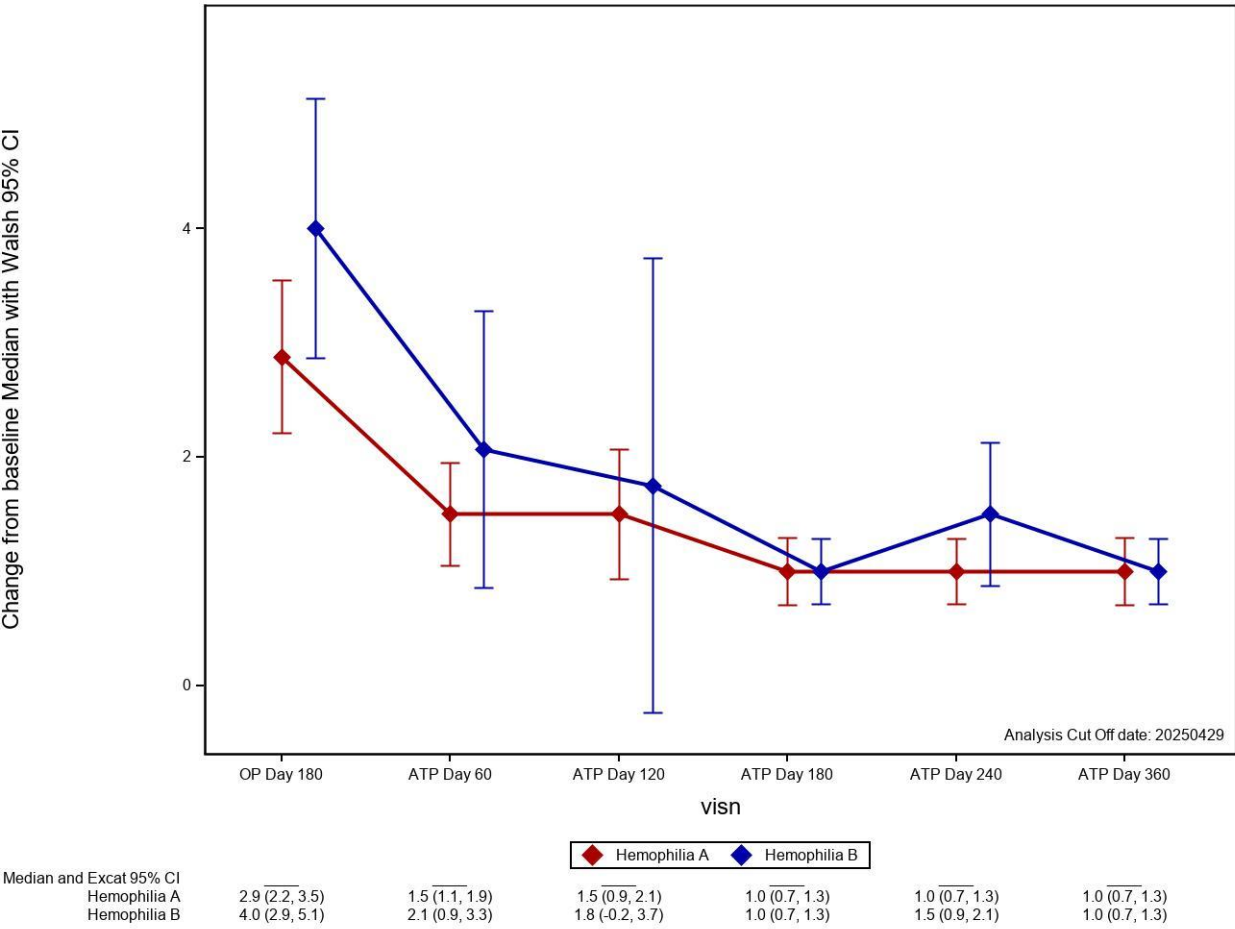
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FIGURE .6.2.44.1: Patient Global Impression of Change (PGIC-H) - PGI01-Improvement - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



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FIGURE .6.2.44.2: Patient Global Impression of Change (PGIC-H) - PGI01-Improvement - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



6 Körperliche Funktionsfähigkeit

6.1 HAL

6.1.1 Hauptanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.1.5. HAL Global score response (≥ 15 points Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Overall								
-	35	5.9 (16.9%)	12.0 (34.3%)	9.3 (26.6%)	2.05 (0.83, 5.06) 0.1200	2.59 (0.81, 8.31) 0.1093	17.4 (-2.9, 37.8) 0.0935	
Disease type								
Hemophilia A	30	5.3 (17.7%)	9.0 (30.0%)	6.3 (21.0%)	1.70 (0.68, 4.29) 0.2574	2.00 (0.61, 6.55) 0.2497	12.3 (-8.2, 32.9) 0.2395	
Hemophilia B	5	0.6 (12.0%)	3.0 (60.0%)	3.0 (60.0%)	3.37 (0.42, 26.90) 0.2524	8.88 (0.29, 270.70) 0.2100	48.0 (-16.4, 112.4) 0.1440	0.4186
Age group								
Adults (Ages 18 to < 75)	35	5.9 (16.9%)	12.0 (34.3%)	9.3 (26.6%)	2.05 (0.83, 5.06) 0.1200	2.59 (0.81, 8.31) 0.1093	17.4 (-2.9, 37.8) 0.0935	-
Region								
Asia	18	2.6 (14.4%)	6.0 (33.3%)	7.3 (40.6%)	-	-	-	
Europe	5	0.0 (0.0%)	2.0 (40.0%)	1.0 (20.0%)	-	-	-	
Other	12	3.3 (27.5%)	4.0 (33.3%)	1.0 (8.3%)	-	-	-	-
White Race								
White	10	2.0 (20.0%)	3.0 (30.0%)	2.0 (20.0%)	1.50 (0.25, 8.98) 0.6569	1.71 (0.16, 18.28) 0.6554	10.0 (-33.4, 53.4) 0.6514	
Other	25	3.9 (15.6%)	9.0 (36.0%)	7.3 (29.2%)	2.35 (0.81, 6.80) 0.1160	3.09 (0.81, 11.77) 0.0978	20.4 (-2.1, 42.9) 0.0758	0.6705

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visis data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD where performed by imputation, and the results were pooled using Rubin's rules.

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In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 10.

Incidences by phase					ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Lying / sitting / kneeling / standing- (Normalized)									
Overall									
Modified Intent-to-Treat Set	35	5 (14.3%)	9 (25.7%)	10 (28.6%)	1.80 (0.60, 5.37) 0.2920		2.08 (0.54, 7.99) 0.2877	11.4 (-9.2, 32.0) 0.2771	
Disease type									
Hemophilia A	30	5 (16.7%)	6 (20.0%)	7 (23.3%)	1.20 (0.37, 3.93) 0.7633		1.25 (0.29, 5.34) 0.7632	3.3 (-18.3, 25.0) 0.7627	
Hemophilia B	5	0 (0.0%)	3 (60.0%)	3 (60.0%)	3.00 (0.31, 28.84) 0.3414		6.00 (0.17, 214.89) 0.3264	40.0 (-30.1, 110.1) 0.2636	0.4052
Region									
Asia	18	2 (11.1%)	6 (33.3%)	6 (33.3%)	-		-	-	
Europe	5	0 (0.0%)	1 (20.0%)	2 (40.0%)	-		-	-	
Other	12	3 (25.0%)	2 (16.7%)	2 (16.7%)	-		-	-	-
White Race									
White	10	1 (10.0%)	2 (20.0%)	3 (30.0%)	2.00 (0.18, 22.06) 0.5714		2.25 (0.14, 36.32) 0.5677	10.0 (-23.4, 43.4) 0.5571	
Other	25	4 (16.0%)	7 (28.0%)	7 (28.0%)	1.75 (0.51, 5.98) 0.3719		2.04 (0.43, 9.65) 0.3679	12.0 (-13.6, 37.6) 0.3577	0.9521

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
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 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 2 of 10.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		
DOMAIN: Functions of the legs-(Normalized)									
Overall									
Set									
Modified Intent-to-Treat	35	4 (11.4%)	16 (45.7%)	13 (37.1%)	4.00 (1.50, 10.66) 0.0056	6.53 (1.93, 22.06) 0.0025	34.3 (15.0, 53.6) 0.0005		
Disease type									
Hemophilia A	30	4 (13.3%)	12 (40.0%)	9 (30.0%)	3.00 (1.13, 7.99) 0.0280	4.33 (1.26, 14.86) 0.0197	26.7 (6.1, 47.2) 0.0109		
Hemophilia B	5	0 (0.0%)	4 (80.0%)	4 (80.0%)	4.00 (0.73, 21.84) 0.1094	16.00 (1.09, 234.25) 0.0429	60.0 (17.1, 102.9) 0.0062	0.3668	
Region									
Asia	18	1 (5.6%)	10 (55.6%)	9 (50.0%)	-	-	-		
Europe	5	0 (0.0%)	2 (40.0%)	2 (40.0%)	-	-	-		
Other	12	3 (25.0%)	4 (33.3%)	2 (16.7%)	-	-	-		-
White Race									
White	10	1 (10.0%)	5 (50.0%)	3 (30.0%)	5.00 (0.87, 28.86) 0.0720	9.00 (1.19, 68.13) 0.0334	40.0 (9.6, 70.4) 0.0098		
Other	25	3 (12.0%)	11 (44.0%)	10 (40.0%)	3.67 (1.12, 11.96) 0.0312	5.76 (1.29, 25.65) 0.0215	32.0 (7.9, 56.1) 0.0092	0.7214	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
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 Datacut: B7841005 (29APR2025)
 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 3 of 10.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI)		OR (95%CI)	RD (95%CI)	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	p-value [1]		p-value [2]	p-value [3]	
DOMAIN: Functions of the arms-(Normalized)									
Overall									
Modified Intent-to-Treat Set	35	9 (25.7%)	15 (42.9%)	10 (28.6%)	1.67 (0.81, 3.41) 0.1618		2.17 (0.75, 6.30) 0.1554	17.1 (-5.9, 40.2) 0.1453	
Disease type									
Hemophilia A	30	9 (30.0%)	13 (43.3%)	8 (26.7%)	1.44 (0.70, 2.98) 0.3200		1.78 (0.58, 5.53) 0.3158	13.3 (-12.4, 39.0) 0.3091	
Hemophilia B	5	0 (0.0%)	2 (40.0%)	2 (40.0%)	3.00 (0.61, 14.86) 0.1785		6.00 (0.67, 53.68) 0.1090	40.0 (-2.9, 82.9) 0.0679	0.3372
Region									
Asia	18	2 (11.1%)	9 (50.0%)	8 (44.4%)	-		-	-	
Europe	5	2 (40.0%)	3 (60.0%)	1 (20.0%)	-		-	-	
Other	12	5 (41.7%)	3 (25.0%)	1 (8.3%)	-		-	-	-
White Race									
White	10	5 (50.0%)	4 (40.0%)	1 (10.0%)	0.80 (0.25, 2.55) 0.7060		0.67 (0.08, 5.45) 0.7054	-10.0 (-61.5, 41.5) 0.7034	
Other	25	4 (16.0%)	11 (44.0%)	9 (36.0%)	2.75 (1.03, 7.33) 0.0431		4.12 (1.13, 15.06) 0.0320	28.0 (4.4, 51.6) 0.0199	0.1655

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
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 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 4 of 10.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI)		OR (95%CI)		RD (95%CI)	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	p-value [1]		p-value [2]		p-value [3]	
DOMAIN: Use of transportation-(Normalized)										
Overall										
Modified Intent-to-Treat Set	35	6 (17.1%)	7 (20.0%)	9 (25.7%)	1.17 (0.43, 3.18)	0.7633	1.21 (0.35, 4.14)	0.7631	2.9 (-15.7, 21.4)	0.7627
Disease type										
Hemophilia A	30	6 (20.0%)	6 (20.0%)	7 (23.3%)	1.00 (0.36, 2.81)	1.0000	1.00 (0.27, 3.64)	1.0000	-0.0 (-20.7, 20.7)	1.0000
Hemophilia B	5	0 (0.0%)	1 (20.0%)	2 (40.0%)	2.00 (0.50, 8.00)	0.3270	2.67 (0.45, 15.96)	0.2826	20.0 (-15.1, 55.1)	0.3968
Region										
Asia	18	1 (5.6%)	4 (22.2%)	7 (38.9%)	-	-	-	-	-	-
Europe	5	1 (20.0%)	1 (20.0%)	0 (0.0%)	-	-	-	-	-	-
Other	12	4 (33.3%)	2 (16.7%)	2 (16.7%)	-	-	-	-	-	-
White Race										
White	10	3 (30.0%)	1 (10.0%)	0 (0.0%)	0.50 (0.13, 2.00)	0.3270	0.38 (0.06, 2.50)	0.3111	-20.0 (-57.2, 17.2)	0.2918
Other	25	3 (12.0%)	6 (24.0%)	9 (36.0%)	2.00 (0.59, 6.79)	0.2663	2.32 (0.54, 9.93)	0.2582	12.0 (-8.2, 32.2)	0.2443

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
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 Datacut: B7841005 (29APR2025)
 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 5 of 10.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		
DOMAIN: Self care-(Normalized)									
Overall									
Modified Intent-to-Treat	35	4 (11.4%)	12 (34.3%)	12 (34.3%)	3.00 (1.04, 8.65) 0.0419	4.04 (1.11, 14.74) 0.0343	22.9 (3.3, 42.4) 0.0218		
Disease type									
Hemophilia A	30	3 (10.0%)	10 (33.3%)	10 (33.3%)	3.33 (0.92, 12.11) 0.0674	4.50 (0.95, 21.36) 0.0584	23.3 (1.3, 45.4) 0.0379		
Hemophilia B	5	1 (20.0%)	2 (40.0%)	2 (40.0%)	2.00 (0.50, 8.00) 0.3270	2.67 (0.45, 15.96) 0.2826	20.0 (-15.1, 55.1) 0.2636	0.6795	
Region									
Asia	18	2 (11.1%)	6 (33.3%)	7 (38.9%)	-	-	-		
Europe	5	0 (0.0%)	2 (40.0%)	1 (20.0%)	-	-	-		
Other	12	2 (16.7%)	4 (33.3%)	4 (33.3%)	-	-	-		-
White Race									
White	10	1 (10.0%)	4 (40.0%)	2 (20.0%)	4.00 (0.45, 35.79) 0.2150	6.00 (0.40, 89.31) 0.1934	30.0 (-9.7, 69.7) 0.1384		
Other	25	3 (12.0%)	8 (32.0%)	10 (40.0%)	2.67 (0.80, 8.86) 0.1092	3.45 (0.80, 14.90) 0.0970	20.0 (-2.2, 42.2) 0.0771	0.7140	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 6 of 10.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Household tasks-(Normalized)										
Overall										
Set										
Modified Intent-to-Treat	35	5 (14.3%)	11 (31.4%)	9 (25.7%)	2.20 (1.04, 4.65)	0.0387	2.75 (1.11, 6.83)	0.0294	17.1 (2.4, 31.9)	0.0231
Disease type										
Hemophilia A	30	5 (16.7%)	10 (33.3%)	7 (23.3%)	2.00 (0.96, 4.16)	0.0640	2.50 (0.99, 6.30)	0.0518	16.7 (0.4, 32.9)	0.0441
Hemophilia B	5	0 (0.0%)	1 (20.0%)	2 (40.0%)	2.00 (0.18, 22.06)	0.5714	2.67 (0.09, 75.80)	0.5658	20.0 (-45.6, 85.6)	0.9708
Region										
Asia	18	1 (5.6%)	4 (22.2%)	5 (27.8%)	-		-		-	
Europe	5	3 (60.0%)	4 (80.0%)	3 (60.0%)	-		-		-	
Other	12	1 (8.3%)	3 (25.0%)	1 (8.3%)	-		-		-	-
White Race										
White	10	3 (30.0%)	5 (50.0%)	4 (40.0%)	1.67 (0.81, 3.41)	0.1618	2.33 (0.79, 6.88)	0.1248	20.0 (-4.8, 44.8)	0.1138
Other	25	2 (8.0%)	6 (24.0%)	5 (20.0%)	3.00 (0.75, 12.00)	0.1203	3.63 (0.76, 17.40)	0.1066	16.0 (-2.2, 34.2)	0.0840
										0.6399

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
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 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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Incidences by phase					ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Leisure activities and sports-(Normalized)									
Overall									
Modified Intent-to-Treat Set	35	7 (20.0%)	11 (31.4%)	8 (22.9%)	1.57 (0.78, 3.18) 0.2098		1.83 (0.72, 4.65) 0.2022	11.4 (-5.9, 28.7) 0.1954	
Disease type									
Hemophilia A	30	7 (23.3%)	10 (33.3%)	7 (23.3%)	1.43 (0.71, 2.88) 0.3199		1.64 (0.62, 4.32) 0.3141	10.0 (-9.3, 29.3) 0.3091	
Hemophilia B	5	0 (0.0%)	1 (20.0%)	1 (20.0%)	2.00 (0.18, 22.06) 0.5714		2.67 (0.09, 75.80) 0.5658	20.0 (-45.6, 85.6) 0.5501	0.7810
Region									
Asia	18	2 (11.1%)	5 (27.8%)	5 (27.8%)	-		-	-	
Europe	5	2 (40.0%)	4 (80.0%)	2 (40.0%)	-		-	-	
Other	12	3 (25.0%)	2 (16.7%)	1 (8.3%)	-		-	-	-
White Race									
White	10	3 (30.0%)	5 (50.0%)	3 (30.0%)	1.67 (0.61, 4.59) 0.3226		2.33 (0.46, 11.83) 0.3062	20.0 (-17.2, 57.2) 0.2918	
Other	25	4 (16.0%)	6 (24.0%)	5 (20.0%)	1.50 (0.56, 4.00) 0.4174		1.66 (0.49, 5.56) 0.4129	8.0 (-10.9, 26.9) 0.4079	0.7406

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
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 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 8 of 10.

Incidences by phase					ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Upper Extremity Activities-(Normalized)									
Overall									
Modified Intent-to-Treat	35	4 (11.4%)	12 (34.3%)	9 (25.7%)	3.00 (1.13, 7.99) 0.0280		4.04 (1.24, 13.19) 0.0206	22.9 (5.0, 40.7) 0.0121	
Set									
Disease type									
Hemophilia A	30	4 (13.3%)	10 (33.3%)	7 (23.3%)	2.50 (0.94, 6.66) 0.0669		3.25 (0.97, 10.89) 0.0561	20.0 (0.6, 39.4) 0.0431	
Hemophilia B	5	0 (0.0%)	2 (40.0%)	2 (40.0%)	3.00 (0.61, 14.86) 0.1785		6.00 (0.67, 53.68) 0.1090	40.0 (-2.9, 82.9) 0.0679	0.6246
Region									
Asia	18	1 (5.6%)	6 (33.3%)	7 (38.9%)	-		-	-	
Europe	5	0 (0.0%)	2 (40.0%)	1 (20.0%)	-		-	-	
Other	12	3 (25.0%)	4 (33.3%)	1 (8.3%)	-		-	-	-
White Race									
White	10	2 (20.0%)	5 (50.0%)	2 (20.0%)	2.50 (0.63, 10.00) 0.1950		4.00 (0.55, 29.10) 0.1709	30.0 (-9.7, 69.7) 0.1384	
Other	25	2 (8.0%)	7 (28.0%)	7 (28.0%)	3.50 (0.88, 13.99) 0.0764		4.47 (0.92, 21.64) 0.0626	20.0 (0.8, 39.2) 0.0412	0.9313

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
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 TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Basic Lower Extremity Activities-(Normalized)										
Overall										
Modified Intent-to-Treat Set	35	5 (14.3%)	17 (48.6%)	13 (37.1%)	3.40 (1.53, 7.53) 0.0026		5.67 (2.07, 15.51) 0.0007		34.3 (16.7, 51.9) 0.0001	
Disease type										
Hemophilia A	30	4 (13.3%)	13 (43.3%)	9 (30.0%)	3.25 (1.32, 8.01) 0.0104		4.97 (1.63, 15.19) 0.0049		30.0 (11.2, 48.8) 0.0018	
Hemophilia B	5	1 (20.0%)	4 (80.0%)	4 (80.0%)	4.00 (0.73, 21.84) 0.1094		16.00 (1.09, 234.25) 0.0429		60.0 (17.1, 102.9) 0.0062	0.4072
Region										
Asia	18	2 (11.1%)	9 (50.0%)	9 (50.0%)	-		-		-	
Europe	5	0 (0.0%)	3 (60.0%)	1 (20.0%)	-		-		-	
Other	12	3 (25.0%)	5 (41.7%)	3 (25.0%)	-		-		-	-
White Race										
White	10	2 (20.0%)	6 (60.0%)	2 (20.0%)	3.00 (0.97, 9.30) 0.0571		6.00 (1.27, 28.25) 0.0234		40.0 (9.6, 70.4) 0.0098	
Other	25	3 (12.0%)	11 (44.0%)	11 (44.0%)	3.67 (1.25, 10.79) 0.0183		5.76 (1.53, 21.69) 0.0096		32.0 (10.6, 53.4) 0.0034	0.9689

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

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TABLE 4.2.3. HAL response (≥ 15 points Improvement from baseline) analysis

Modified Intent-to-Treat Set

Table page 10 of 10.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
DOMAIN: Complex Lower Extremity Activities-(Normalized)										
Overall										
Set										
Modified Intent-to-Treat	35	4 (11.4%)	7 (20.0%)	8 (22.9%)	1.75 (0.51, 5.98) 0.3719	1.94 (0.46, 8.20) 0.3687	8.6 (-9.8, 26.9) 0.3600			
Disease type										
Hemophilia A	30	4 (13.3%)	5 (16.7%)	5 (16.7%)	1.25 (0.34, 4.65) 0.7394	1.30 (0.28, 6.09) 0.7391	3.3 (-16.2, 22.9) 0.7384			
Hemophilia B	5	0 (0.0%)	2 (40.0%)	3 (60.0%)	2.00 (0.18, 22.06) 0.5714	2.67 (0.09, 75.80) 0.5658	20.0 (-45.6, 85.6) 0.5501	0.6978		
Region										
Asia	18	1 (5.6%)	5 (27.8%)	5 (27.8%)	-	-	-			
Europe	5	0 (0.0%)	1 (20.0%)	1 (20.0%)	-	-	-			
Other	12	3 (25.0%)	1 (8.3%)	2 (16.7%)	-	-	-			-
White Race										
White	10	1 (10.0%)	2 (20.0%)	3 (30.0%)	2.00 (0.18, 22.06) 0.5714	2.25 (0.14, 36.32) 0.5677	10.0 (-23.4, 43.4) 0.5571			
Other	25	3 (12.0%)	5 (20.0%)	5 (20.0%)	1.67 (0.40, 6.97) 0.4843	1.83 (0.34, 9.92) 0.4818	8.0 (-14.0, 30.0) 0.4751	0.9010		

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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6.1.2 Sensitivitätsanalyse

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 Datacut: B7841005 (29APR2025)
 TABLE 6.1.25. Hemophilia Activities List for Adults (HAL) - Total Score-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 51.7 (19.85) 38.0 48.39 70.3	-	-
OP Day 180	n=32 50.5 (23.36) 36.5 46.90 66.0	n=31 0.7 (17.29) -8.0 0.17 7.0	0.4 (-5.2, 5.9)
ATP Baseline	n=35 51.7 (23.59) 37.6 48.10 68.6	-	-
ATP Day 180	n=33 59.4 (22.34) 48.4 60.00 72.4	n=33 9.9 (20.56) 1.0 11.43 21.4	10.6 (4.8, 16.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	10.4 (2.7, 18.2)
ATP Day 360	n=34 58.7 (22.96) 42.9 60.25 75.7	n=34 7.3 (20.64) -0.5 5.00 16.6	0.0085 7.1 (1.8, 12.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.25. Hemophilia Activities List for Adults (HAL) - Total Score- (Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 52.3 (20.11) 38.0 49.05 70.3	-	-
OP Day 180	n=27 50.6 (24.42) 37.4 47.14 65.2	n=27 1.4 (18.42) -8.0 1.43 11.0	0.5 (-6.8, 7.8)
ATP Baseline	n=30 52.0 (24.67) 37.8 49.31 68.6	-	-
ATP Day 180	n=28 58.2 (23.66) 45.9 59.76 75.2	n=28 8.7 (22.00) -0.9 10.88 21.6	9.4 (1.7, 17.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	9.1 (-1.1, 19.4)
ATP Day 360	n=29 57.0 (24.03) 39.9 59.44 68.6	n=29 5.3 (20.85) -0.5 4.38 12.0	0.0750 5.2 (-0.7, 11.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.25. Hemophilia Activities List for Adults (HAL) - Total Score-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 47.9 (20.08) 34.3 42.62 61.4	-	-
OP Day 180	n=5 49.6 (18.81) 34.3 46.67 66.7	n=4 -3.6 (4.35) -6.9 -2.51 -0.4	-1.6 (-33.5, 30.2)
ATP Baseline	n=5 49.9 (17.63) 37.6 44.62 63.4	-	-
ATP Day 180	n=5 66.4 (11.87) 58.6 68.57 72.4	n=5 16.5 (7.20) 9.0 20.00 21.0	16.3 (7.5, 25.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	21.0 (-14.1, 56.2)
ATP Day 360	n=5 69.1 (12.22) 70.0 75.43 75.7	n=5 19.2 (16.30) 12.3 16.64 30.8	0.2122 18.0 (-5.7, 41.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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Inhibitor with On-Demand at OP

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TABLE 6.1.26. Hemophilia Activities List for Adults (HAL) - Lying / sitting / kneeling / standing-(Normalized).

Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 43.6 (20.90) 27.5 42.50 55.0	-	-
OP Day 180	n=32 42.2 (23.37) 31.3 37.50 56.3	n=31 0.3 (15.90) -10.0 0.00 12.5	0.6 (-7.5, 7.5)
ATP Baseline	n=35 42.3 (23.79) 27.5 37.50 57.5	-	-
ATP Day 180	n=33 45.9 (20.83) 32.5 47.50 60.0	n=33 5.6 (23.01) 0.0 5.00 17.5	8.4 (1.9, 16.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	9.1 (-2.5, 20.0)
ATP Day 360	n=34 48.4 (21.45) 32.5 47.50 60.0	n=34 6.5 (17.88) 0.0 6.25 15.0	0.1028 8.1 (0.0, 16.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.26. Hemophilia Activities List for Adults (HAL) - Lying / sitting / kneeling / standing-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 44.0 (21.33) 27.5 43.75 55.0	-	-
OP Day 180	n=27 42.2 (24.88) 32.5 37.50 57.5	n=27 0.6 (16.68) -10.0 0.00 12.5	1.0 (-7.6, 9.6)
ATP Baseline	n=30 43.8 (24.75) 27.5 38.75 57.5	-	-
ATP Day 180	n=28 45.2 (22.11) 30.6 45.00 60.0	n=28 3.6 (24.19) -1.3 5.00 13.8	5.5 (-3.1, 14.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	5.1 (-10.3, 20.5)
ATP Day 360	n=29 47.7 (22.45) 32.5 47.50 57.5	n=29 4.3 (17.51) 0.0 2.50 12.5	0.4755 7.4 (-1.3, 16.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
Marstacimab

Inhibitor with On-Demand at OP

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TABLE 6.1.26. Hemophilia Activities List for Adults (HAL) - Lying / sitting / kneeling / standing-(Normalized)

Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 40.6 (19.83) 28.8 32.50 52.5	-	-
OP Day 180	n=5 42.0 (14.51) 30.0 42.50 55.0	n=4 -1.9 (10.48) -8.8 -3.75 5.0	3.8 (-33.9, 41.4)
ATP Baseline	n=5 33.5 (16.07) 22.5 25.00 42.5	-	-
ATP Day 180	n=5 50.0 (12.12) 45.0 50.00 60.0	n=5 16.5 (10.55) 10.0 20.00 20.0	16.3 (0.6, 31.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	15.0 (-18.4, 48.4)
ATP Day 360	n=5 52.5 (15.61) 45.0 55.00 62.5	n=5 19.0 (16.16) 7.5 25.00 27.5	0.3408 17.5 (-5.2, 40.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.27. Hemophilia Activities List for Adults (HAL) - Functions of the legs-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 42.2 (26.88) 24.4 41.11 53.3	-	-
OP Day 180	n=32 42.2 (27.26) 23.3 41.11 56.7	n=31 2.6 (16.56) -11.1 2.22 8.9	2.8 (-4.4, 8.9)
ATP Baseline	n=35 43.8 (29.44) 17.8 42.22 64.4	-	-
ATP Day 180	n=33 51.1 (28.62) 28.9 53.33 68.9	n=33 9.6 (23.52) 0.0 13.33 24.4	16.7 (5.6, 24.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	11.1 (2.2, 21.1) 0.0200
ATP Day 360	n=34 51.6 (28.64) 28.9 58.89 68.9	n=34 8.1 (23.99) -2.2 6.67 24.4	8.9 (-0.6, 18.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.27. Hemophilia Activities List for Adults (HAL) - Functions of the legs-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 42.1 (27.42) 22.2 45.56 53.3	-	-
OP Day 180	n=27 42.0 (28.37) 22.2 40.00 55.6	n=27 3.8 (16.73) -8.9 4.44 8.9	4.1 (-4.4, 12.5)
ATP Baseline	n=30 44.5 (30.81) 17.8 43.33 64.4	-	-
ATP Day 180	n=28 49.4 (30.54) 26.7 51.11 70.0	n=28 7.5 (24.62) 0.0 12.22 24.4	11.9 (-0.0, 23.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	6.3 (-5.8, 18.5) 0.2736
ATP Day 360	n=29 50.0 (30.34) 26.7 57.78 68.9	n=29 5.9 (24.38) -2.2 4.44 20.0	8.1 (-2.7, 18.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.27. Hemophilia Activities List for Adults (HAL) - Functions of the legs-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 43.3 (26.07) 28.9 32.22 57.8	-	-
OP Day 180	n=5 43.1 (22.91) 28.9 42.22 64.4	n=4 -5.6 (14.68) -17.8 -6.67 6.7	0.6 (-22.8, 23.9)
ATP Baseline	n=5 39.6 (21.45) 26.7 31.11 51.1	-	-
ATP Day 180	n=5 60.9 (10.73) 53.3 55.56 68.9	n=5 21.3 (11.40) 17.8 24.44 24.4	21.1 (3.4, 38.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	20.0 (-13.5, 53.5)
ATP Day 360	n=5 60.9 (14.02) 62.2 62.22 66.7	n=5 21.3 (18.23) 20.0 24.44 35.6	0.2127 24.4 (-0.8, 49.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.28. Hemophilia Activities List for Adults (HAL) - Functions of the arms-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 56.6 (22.89) 40.0 55.00 70.0	-	-
OP Day 180	n=32 55.6 (26.93) 35.0 60.00 75.0	n=31 2.1 (26.54) -10.0 0.00 15.0	1.2 (-12.5, 15.0)
ATP Baseline	n=35 57.9 (26.60) 40.0 60.00 80.0	-	-
ATP Day 180	n=33 63.0 (26.43) 45.0 60.00 85.0	n=33 7.1 (22.12) 0.0 10.00 20.0	11.2 (0.0, 20.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	7.5 (-10.0, 22.5)
ATP Day 360	n=34 59.7 (30.17) 40.0 67.50 80.0	n=34 2.1 (25.88) -15.0 0.00 20.0	0.2515 6.2 (-7.5, 17.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 6.1.28. Hemophilia Activities List for Adults (HAL) - Functions of the arms-(Normalized)

Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 56.5 (23.49) 40.0 55.00 70.0	-	-
OP Day 180	n=27 54.6 (27.24) 35.0 60.00 70.0	n=27 3.0 (28.29) -10.0 0.00 25.0	0.9 (-12.6, 14.4)
ATP Baseline	n=30 56.7 (27.27) 35.0 60.00 80.0	-	-
ATP Day 180	n=28 61.1 (27.67) 40.0 60.00 82.5	n=28 6.8 (23.85) -5.0 12.50 22.5	10.7 (-2.4, 23.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	6.1 (-12.8, 24.9)
ATP Day 360	n=29 56.0 (31.09) 35.0 60.00 80.0	n=29 -0.3 (25.91) -20.0 0.00 15.0	0.4907 -0.3 (-15.9, 15.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
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TABLE 6.1.28. Hemophilia Activities List for Adults (HAL) - Functions of the arms-(Normalized)
Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 57.5 (20.62) 40.0 55.00 75.0	-	-
OP Day 180	n=5 61.0 (27.48) 40.0 70.00 80.0	n=4 -3.8 (7.50) -7.5 0.00 0.0	21.8 (-20.0, 63.6)
ATP Baseline	n=5 65.0 (23.45) 40.0 75.00 80.0	-	-
ATP Day 180	n=5 74.0 (15.57) 60.0 80.00 85.0	n=5 9.0 (8.22) 5.0 5.00 15.0	11.3 (2.7, 19.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	8.8 (-38.7, 56.2)
ATP Day 360	n=5 81.0 (8.94) 75.0 80.00 90.0	n=5 16.0 (23.29) 0.0 5.00 30.0	0.6900 20.0 (-11.3, 51.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.
Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).
Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.29. Hemophilia Activities List for Adults (HAL) - Use of transportation-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=31 51.2 (25.86) 26.7 53.33 66.7	-	-
OP Day 180	n=29 49.5 (30.26) 20.0 46.67 73.3	n=26 0.4 (25.20) -13.3 -3.33 13.3	-3.3 (-13.3, 10.0)
ATP Baseline	n=28 52.6 (26.93) 40.0 50.00 66.7	-	-
ATP Day 180	n=28 54.4 (27.93) 33.3 60.00 71.7	n=25 7.7 (21.51) 0.0 6.67 20.0	10.0 (-5.0, 26.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	10.0 (-8.3, 25.0)
ATP Day 360	n=30 58.2 (28.53) 40.0 60.00 80.0	n=25 10.0 (22.19) 0.0 6.67 26.7	0.1385 13.3 (-3.3, 25.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.29. Hemophilia Activities List for Adults (HAL) - Use of transportation-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=28 50.5 (24.46) 33.3 50.00 66.7	-	-
OP Day 180	n=25 48.7 (29.66) 20.0 40.00 73.3	n=23 2.8 (25.24) -13.3 0.00 20.0	-1.1 (-14.3, 12.0)
ATP Baseline	n=24 51.9 (28.01) 40.0 50.00 66.7	-	-
ATP Day 180	n=24 52.5 (28.86) 31.7 60.00 70.0	n=21 7.5 (22.80) 0.0 0.00 20.0	3.0 (-14.5, 20.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	5.2 (-13.1, 23.5)
ATP Day 360	n=26 56.6 (30.27) 40.0 50.00 86.7	n=21 9.7 (21.96) 0.0 6.67 20.0	0.5378 10.7 (-6.3, 27.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.29. Hemophilia Activities List for Adults (HAL) - Use of transportation-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=3 57.8 (43.38) 13.3 60.00 100.0	-	-
OP Day 180	n=4 55.0 (38.25) 30.0 56.67 80.0	n=3 -17.8 (19.25) -40.0 -6.67 -6.7	-3.9 (-60.1, 52.2)
ATP Baseline	n=4 56.7 (22.11) 40.0 50.00 73.3	-	-
ATP Day 180	n=4 65.8 (20.79) 50.0 61.67 81.7	n=4 9.2 (15.24) -0.0 6.67 18.3	9.7 (-12.0, 31.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	19.9 (-46.5, 86.4)
ATP Day 360	n=4 68.3 (8.39) 63.3 66.67 73.3	n=4 11.7 (26.87) -10.0 13.33 33.3	0.5187 26.7 (-27.9, 81.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.30. Hemophilia Activities List for Adults (HAL) - Self care-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 75.3 (23.44) 60.0 78.00 100.0	-	-
OP Day 180	n=32 68.8 (26.11) 52.0 72.00 94.0	n=31 -6.1 (26.71) -16.0 0.00 8.0	-5.0 (-16.0, 4.0)
ATP Baseline	n=35 70.2 (22.59) 56.0 72.00 88.0	-	-
ATP Day 180	n=33 78.5 (23.70) 60.0 88.00 100.0	n=33 9.7 (28.74) -4.0 4.00 20.0	9.0 (0.0, 20.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	15.0 (4.0, 30.0) 0.0056
ATP Day 360	n=34 76.5 (24.55) 64.0 84.00 100.0	n=34 6.2 (28.76) 0.0 8.00 24.0	9.0 (-2.0, 18.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.30. Hemophilia Activities List for Adults (HAL) - Self care-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 75.5 (22.74) 60.0 78.00 100.0	-	-
OP Day 180	n=27 66.2 (26.35) 52.0 64.00 92.0	n=27 -7.6 (28.12) -16.0 0.00 8.0	-6.8 (-19.2, 5.5)
ATP Baseline	n=30 69.3 (22.88) 56.0 70.00 88.0	-	-
ATP Day 180	n=28 75.9 (24.57) 60.0 80.00 100.0	n=28 8.1 (29.56) -8.0 4.00 22.0	7.8 (-3.2, 18.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	14.9 (-0.0, 29.9)
ATP Day 360	n=29 73.8 (25.49) 60.0 72.00 100.0	n=29 4.4 (29.94) 0.0 8.00 16.0	0.0504 9.4 (-4.2, 23.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.30. Hemophilia Activities List for Adults (HAL) - Self care-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 74.0 (32.25) 52.0 84.00 96.0	-	-
OP Day 180	n=5 82.4 (22.20) 72.0 96.00 96.0	n=4 4.0 (11.31) -4.0 0.00 12.0	8.0 (-9.1, 25.1)
ATP Baseline	n=5 75.2 (22.52) 68.0 80.00 92.0	-	-
ATP Day 180	n=5 93.6 (9.21) 88.0 100.00 100.0	n=5 18.4 (24.43) 4.0 8.00 20.0	17.0 (-14.8, 48.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	8.0 (-29.5, 45.5) 0.6449
ATP Day 360	n=5 92.0 (8.49) 88.0 92.00 100.0	n=5 16.8 (19.68) 4.0 8.00 24.0	20.0 (-5.0, 45.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.31. Hemophilia Activities List for Adults (HAL) - Household tasks-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=32 60.6 (29.37) 40.0 61.67 81.7	-	-
OP Day 180	n=30 58.1 (30.32) 36.7 63.33 80.0	n=27 -1.9 (27.95) -10.0 0.00 13.3	-3.0 (-16.7, 10.0)
ATP Baseline	n=33 61.6 (28.75) 40.0 66.67 86.7	-	-
ATP Day 180	n=30 71.1 (29.18) 60.0 80.00 93.3	n=29 12.0 (29.89) 0.0 10.00 30.0	16.7 (5.0, 28.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	20.4 (9.3, 34.3) 0.0017
ATP Day 360	n=31 71.3 (27.04) 60.0 74.00 96.7	n=30 8.7 (27.52) 0.0 8.33 23.3	16.7 (6.7, 30.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.31. Hemophilia Activities List for Adults (HAL) - Household tasks-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=28 62.7 (30.39) 46.7 63.33 90.0	-	-
OP Day 180	n=25 61.2 (30.55) 36.7 68.00 90.0	n=23 -0.7 (29.57) -10.0 0.00 13.3	-3.2 (-17.5, 11.0)
ATP Baseline	n=28 62.3 (30.46) 38.3 66.67 88.3	-	-
ATP Day 180	n=25 71.3 (31.45) 63.3 80.00 96.7	n=24 11.8 (32.20) 0.0 8.33 31.7	14.0 (-1.8, 29.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	18.4 (0.7, 36.1) 0.0430
ATP Day 360	n=26 70.8 (28.55) 60.0 73.67 96.7	n=25 7.1 (28.85) 0.0 3.33 20.0	14.8 (-2.2, 31.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.31. Hemophilia Activities List for Adults (HAL) - Household tasks-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 45.8 (16.41) 36.7 40.00 55.0	-	-
OP Day 180	n=5 42.7 (26.60) 40.0 43.33 63.3	n=4 -9.2 (16.64) -20.0 -3.33 1.7	-4.0 (-34.7, 26.8)
ATP Baseline	n=5 57.3 (18.01) 40.0 63.33 70.0	-	-
ATP Day 180	n=5 70.0 (15.46) 56.7 80.00 80.0	n=5 12.7 (17.06) 6.7 10.00 13.3	10.0 (-16.5, 36.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	14.6 (-14.3, 43.5) 0.2871
ATP Day 360	n=5 74.0 (19.49) 66.7 76.67 80.0	n=5 16.7 (20.00) 10.0 10.00 36.7	13.3 (-13.2, 39.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.32. Hemophilia Activities List for Adults (HAL) - Leisure activities and sports-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=30 48.1 (24.68) 30.0 48.57 65.7	-	-
OP Day 180	n=28 49.9 (28.12) 31.0 51.43 68.3	n=23 5.4 (27.89) -8.0 2.86 18.3	4.3 (-6.9, 17.1)
ATP Baseline	n=28 50.9 (27.45) 34.3 48.57 70.5	-	-
ATP Day 180	n=28 65.0 (28.70) 51.4 74.57 88.3	n=24 16.0 (25.49) 0.0 11.43 36.7	18.5 (6.2, 30.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	11.4 (-1.8, 28.5)
ATP Day 360	n=29 60.6 (29.61) 37.1 62.86 80.0	n=26 9.8 (26.00) 0.0 9.29 17.1	0.0854 10.3 (0.5, 25.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.32. Hemophilia Activities List for Adults (HAL) - Leisure activities and sports-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=26 50.3 (25.24) 33.3 52.86 68.6	-	-
OP Day 180	n=23 52.0 (29.55) 37.1 51.43 71.4	n=19 6.3 (30.65) -13.3 0.00 20.0	4.0 (-11.0, 19.0)
ATP Baseline	n=23 52.3 (27.86) 34.3 54.29 76.0	-	-
ATP Day 180	n=24 65.8 (28.81) 51.4 74.57 88.3	n=20 16.8 (27.59) -1.4 14.29 39.5	17.6 (2.0, 33.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	11.9 (-8.4, 32.1)
ATP Day 360	n=25 59.2 (29.33) 37.1 60.00 80.0	n=22 7.8 (25.98) -2.9 5.71 17.1	0.2210 7.7 (-6.8, 22.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.32. Hemophilia Activities List for Adults (HAL) - Leisure activities and sports-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 33.8 (16.16) 24.0 29.00 43.6	-	-
OP Day 180	n=5 40.4 (19.81) 25.7 33.33 60.0	n=4 1.0 (6.11) -2.6 3.10 4.5	4.3 (-11.6, 20.2)
ATP Baseline	n=5 44.6 (27.47) 30.0 40.00 62.9	-	-
ATP Day 180	n=4 60.7 (31.93) 35.7 65.71 85.7	n=4 12.5 (11.86) 5.0 10.71 20.0	11.4 (0.8, 21.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	7.8 (-18.2, 33.8)
ATP Day 360	n=4 69.3 (34.39) 48.6 78.57 90.0	n=4 21.1 (26.63) 5.0 12.14 37.1	0.5196 17.1 (-11.4, 45.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.33. Hemophilia Activities List for Adults (HAL) - Upper Extremity Activities-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 67.0 (19.63) 53.3 65.56 86.7	-	-
OP Day 180	n=32 62.9 (25.18) 46.7 66.67 82.2	n=31 -2.4 (22.83) -11.1 -2.22 11.1	-0.6 (-7.8, 5.6)
ATP Baseline	n=35 64.7 (21.70) 46.7 66.67 80.0	-	-
ATP Day 180	n=33 71.6 (22.10) 60.0 75.56 86.7	n=33 8.6 (20.04) 0.0 4.44 20.0	11.1 (3.3, 17.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	13.3 (2.2, 22.2) 0.0193
ATP Day 360	n=34 69.0 (24.23) 48.9 75.56 88.9	n=34 4.4 (24.25) -6.7 3.33 15.6	6.7 (-2.2, 15.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.33. Hemophilia Activities List for Adults (HAL) - Upper Extremity Activities-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 67.0 (19.26) 53.3 65.56 82.2	-	-
OP Day 180	n=27 61.1 (25.34) 46.7 62.22 77.8	n=27 -2.9 (24.47) -11.1 -2.22 11.1	-2.9 (-11.9, 6.0)
ATP Baseline	n=30 63.7 (21.85) 46.7 66.67 80.0	-	-
ATP Day 180	n=28 69.3 (22.99) 54.4 73.33 86.7	n=28 7.5 (20.75) -1.1 5.56 20.0	8.9 (-0.2, 18.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	12.0 (-1.0, 25.0)
ATP Day 360	n=29 65.9 (24.85) 46.7 66.67 86.7	n=29 2.3 (24.48) -6.7 2.22 13.3	0.0667 4.2 (-6.1, 14.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.33. Hemophilia Activities List for Adults (HAL) - Upper Extremity Activities-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 66.7 (25.53) 46.7 73.33 86.7	-	-
OP Day 180	n=5 72.9 (24.31) 57.8 84.44 88.9	n=4 0.6 (3.33) -2.2 -0.00 3.3	2.2 (-33.7, 38.1)
ATP Baseline	n=5 70.7 (22.19) 55.6 77.78 88.9	-	-
ATP Day 180	n=5 84.9 (8.94) 80.0 80.00 93.3	n=5 14.2 (16.07) 4.4 4.44 20.0	12.2 (-9.3, 33.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	6.7 (-46.9, 60.3)
ATP Day 360	n=5 87.1 (6.17) 82.2 88.89 88.9	n=5 16.4 (21.06) 2.2 4.44 26.7	0.7873 20.6 (-6.0, 47.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.34. Hemophilia Activities List for Adults (HAL) - Basic Lower Extremity Activities-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 48.0 (28.23) 30.0 50.00 73.3	-	-
OP Day 180	n=32 48.0 (28.32) 30.0 50.00 70.0	n=31 1.9 (15.61) -10.0 0.00 6.7	0.8 (-6.7, 11.7)
ATP Baseline	n=35 47.9 (28.45) 30.0 46.67 73.3	-	-
ATP Day 180	n=33 58.0 (29.86) 43.3 66.67 80.0	n=33 11.8 (27.50) 0.0 16.67 30.0	18.3 (5.0, 28.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	15.0 (1.7, 26.7)
ATP Day 360	n=34 58.0 (30.17) 36.7 60.00 80.0	n=34 10.8 (28.63) -3.3 6.67 26.7	0.0217 13.3 (0.0, 26.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.34. Hemophilia Activities List for Adults (HAL) - Basic Lower Extremity Activities-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 48.2 (29.40) 26.7 53.33 73.3	-	-
OP Day 180	n=27 47.5 (29.32) 26.7 50.00 73.3	n=27 2.2 (15.80) -10.0 0.00 6.7	2.0 (-8.5, 12.6)
ATP Baseline	n=30 48.7 (29.63) 30.0 50.00 73.3	-	-
ATP Day 180	n=28 55.5 (31.72) 38.3 58.33 80.0	n=28 8.8 (28.30) -3.3 13.33 26.7	11.9 (-0.6, 24.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	8.5 (-7.2, 24.3)
ATP Day 360	n=29 55.9 (31.57) 31.7 60.00 80.0	n=29 8.1 (28.70) -3.3 6.67 23.3	0.2561 9.6 (-3.5, 22.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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Inhibitor with On-Demand at OP

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TABLE 6.1.34. Hemophilia Activities List for Adults (HAL) - Basic Lower Extremity Activities-(Normalized)

Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 46.7 (20.18) 35.0 38.33 58.3	-	-
OP Day 180	n=5 50.7 (24.88) 33.3 60.00 66.7	n=4 0.0 (16.33) -10.0 0.00 10.0	10.0 (-12.7, 32.7)
ATP Baseline	n=5 43.3 (21.98) 30.0 30.00 60.0	-	-
ATP Day 180	n=5 72.0 (7.30) 66.7 66.67 80.0	n=5 28.7 (15.02) 20.0 36.67 36.7	28.3 (7.5, 49.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	21.7 (-14.3, 57.7)
ATP Day 360	n=5 70.0 (17.95) 66.7 80.00 80.0	n=5 26.7 (25.06) 16.7 20.00 50.0	0.2096 23.3 (-10.8, 57.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.35. Hemophilia Activities List for Adults (HAL) - Complex Lower Extremity Activities-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 29.7 (23.36) 12.5 22.22 40.0	-	-
OP Day 180	n=32 29.8 (25.76) 12.2 22.22 41.3	n=31 3.4 (19.18) -6.7 0.00 13.3	2.2 (-4.4, 9.3)
ATP Baseline	n=35 31.5 (27.18) 11.1 24.44 50.0	-	-
ATP Day 180	n=33 34.0 (23.52) 17.8 33.33 46.7	n=33 5.1 (19.04) 0.0 8.89 12.8	8.9 (3.1, 13.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	5.8 (-2.5, 13.5) 0.1417
ATP Day 360	n=34 36.5 (26.11) 15.6 35.28 55.6	n=34 5.2 (16.13) -3.6 3.19 11.1	4.3 (-2.0, 11.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.35. Hemophilia Activities List for Adults (HAL) - Complex Lower Extremity Activities-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 29.3 (23.46) 11.1 24.44 40.0	-	-
OP Day 180	n=27 31.0 (27.49) 11.1 24.44 44.4	n=27 5.8 (18.65) -4.4 0.00 13.3	4.3 (-3.4, 12.0)
ATP Baseline	n=30 33.0 (28.66) 11.1 24.72 53.3	-	-
ATP Day 180	n=28 33.7 (25.14) 11.8 31.25 47.1	n=28 3.8 (20.32) -1.4 4.44 11.8	4.9 (-3.2, 13.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	1.5 (-8.6, 11.7) 0.7419
ATP Day 360	n=29 36.3 (27.87) 11.1 35.56 55.6	n=29 3.5 (15.81) -3.6 0.00 8.9	3.4 (-4.1, 10.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 6.1.35. Hemophilia Activities List for Adults (HAL) - Complex Lower Extremity Activities-(Normalized)

Modified Intent-to-Treat Set

Table page 3 of 3.			
Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 32.6 (25.87) 18.6 22.22 46.7	-	-
OP Day 180	n=5 23.4 (13.20) 13.3 15.00 35.6	n=4 -13.3 (15.40) -22.2 -8.89 -4.4	-23.9 (-42.9, -4.9)
ATP Baseline	n=5 22.6 (14.51) 12.5 17.78 25.0	-	-
ATP Day 180	n=5 35.2 (12.44) 24.4 33.33 44.4	n=5 12.6 (5.69) 10.0 13.33 15.6	12.8 (4.3, 21.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	31.1 (1.7, 60.5)
ATP Day 360	n=5 37.2 (13.66) 26.7 35.00 37.8	n=5 14.6 (16.31) 8.9 15.56 22.5	0.0401 15.6 (-9.4, 40.5)

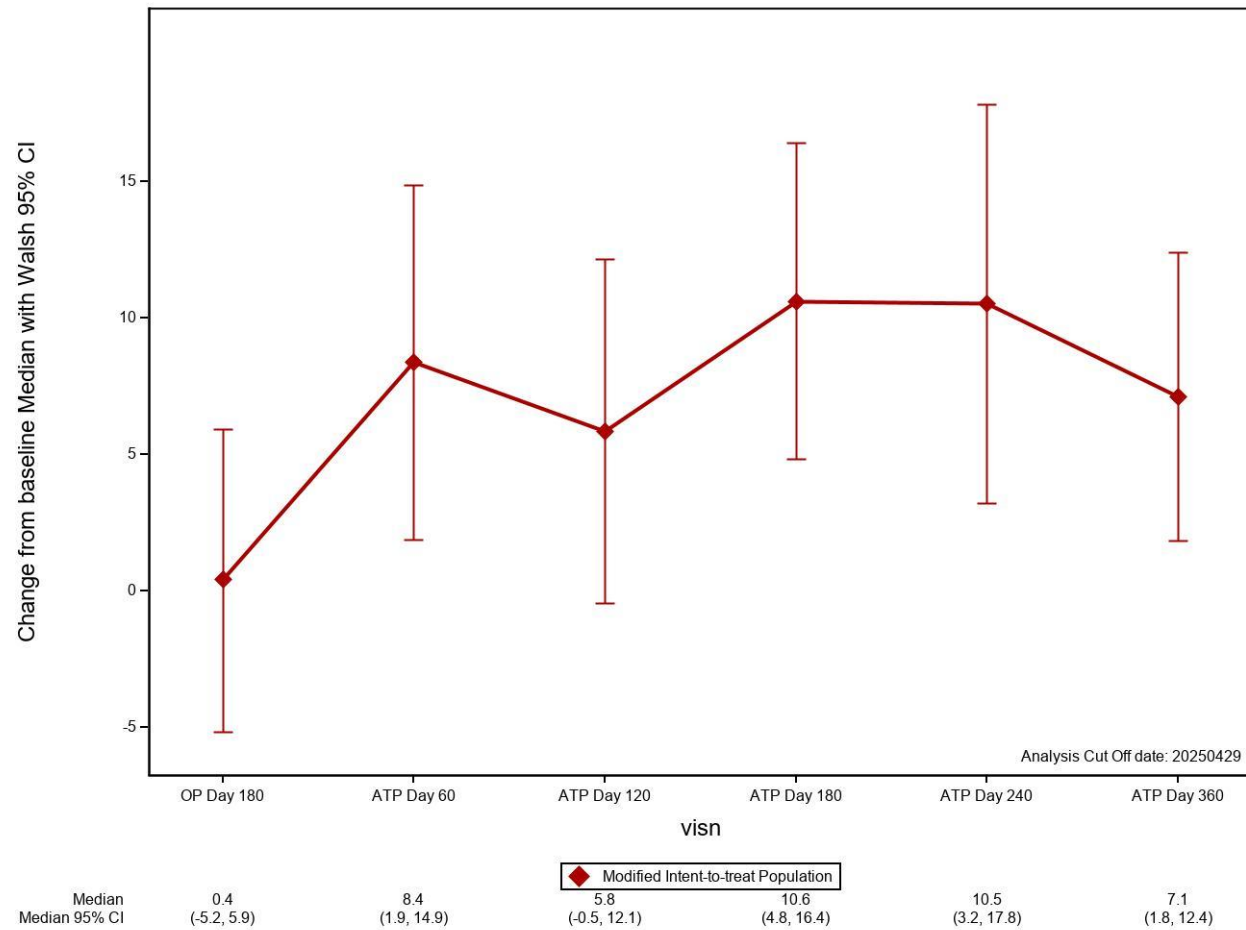
ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

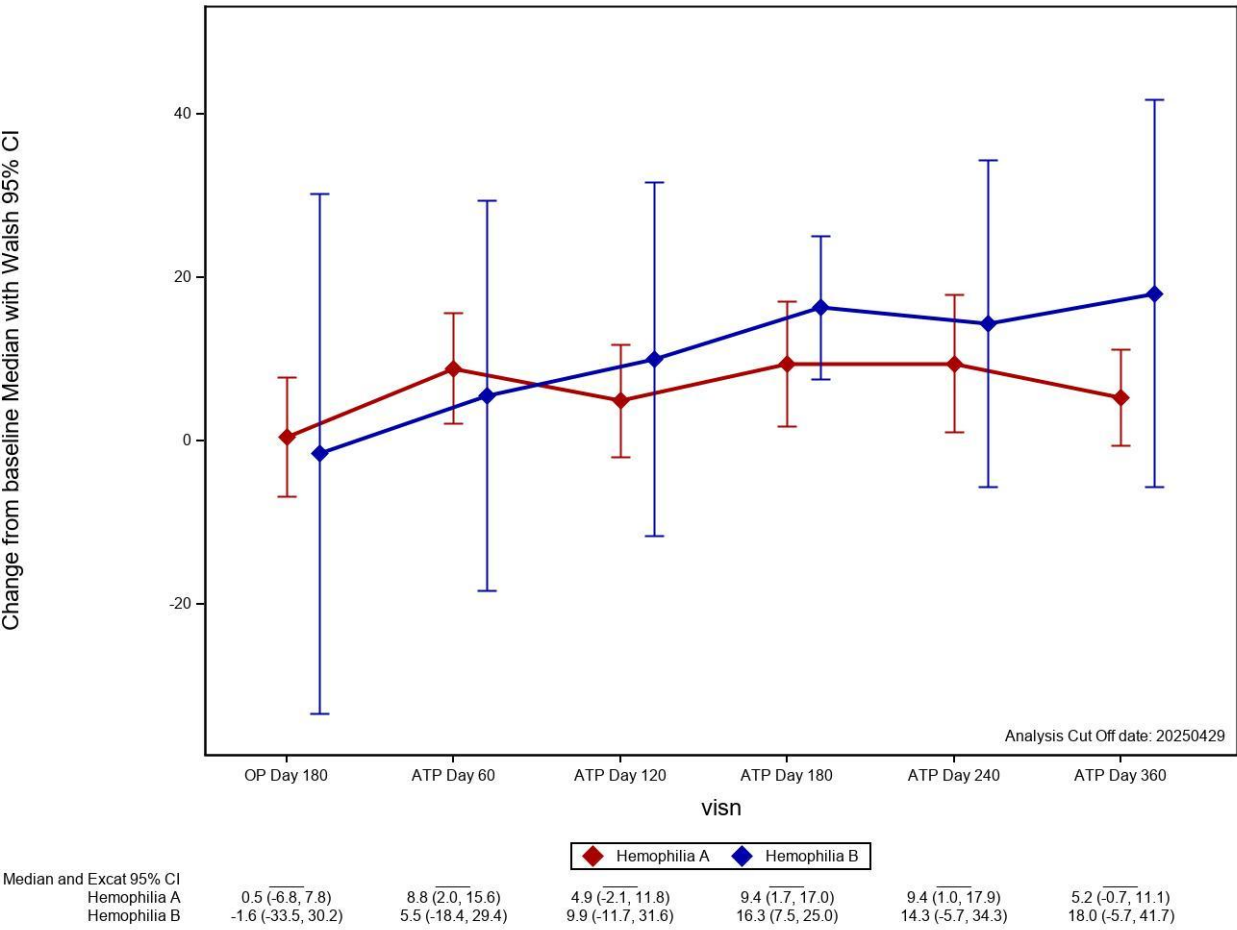
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.25.1: Hemophilia Activities List for Adults (HAL) - Total Score-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



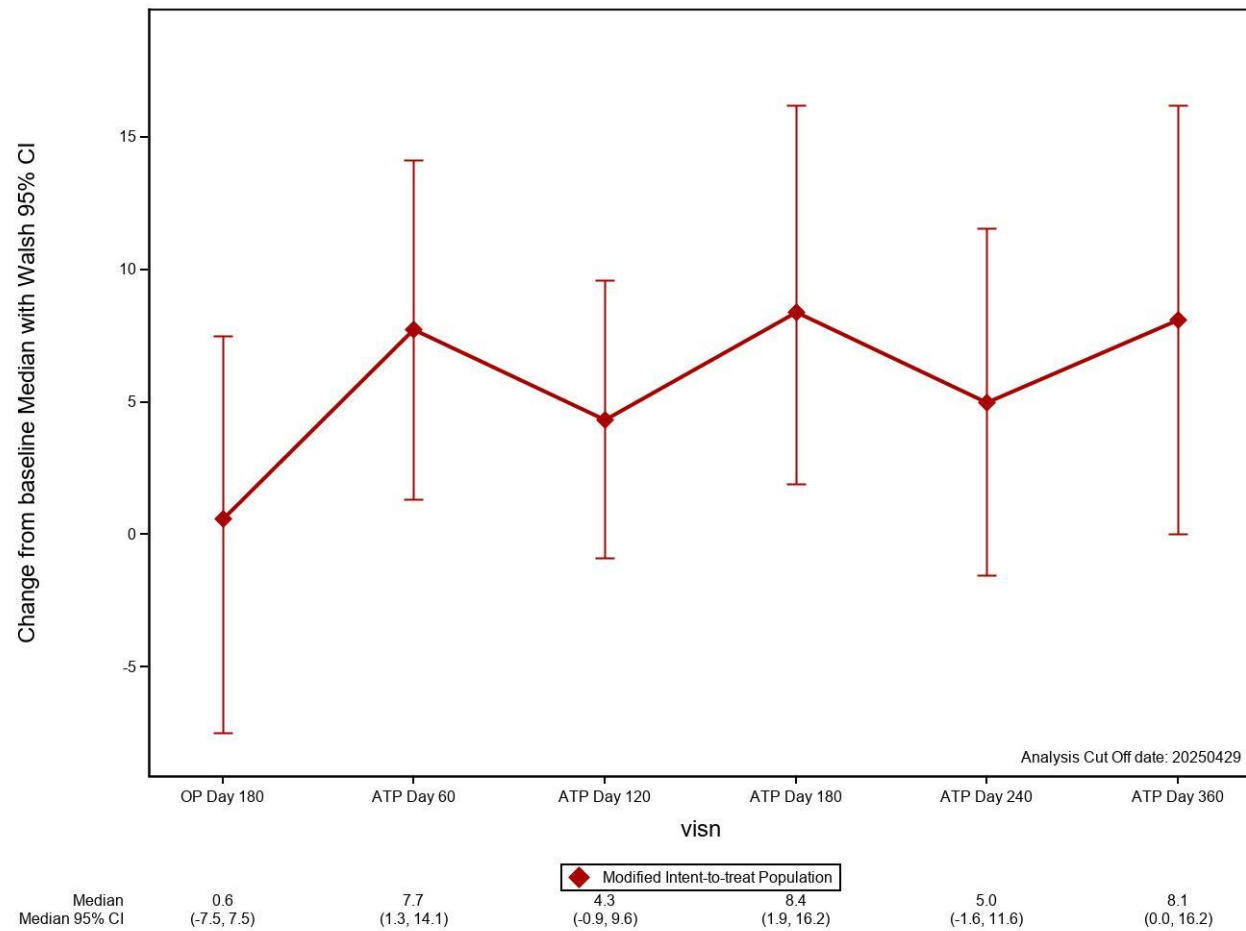
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.25.2: Hemophilia Activities List for Adults (HAL) - Total Score-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



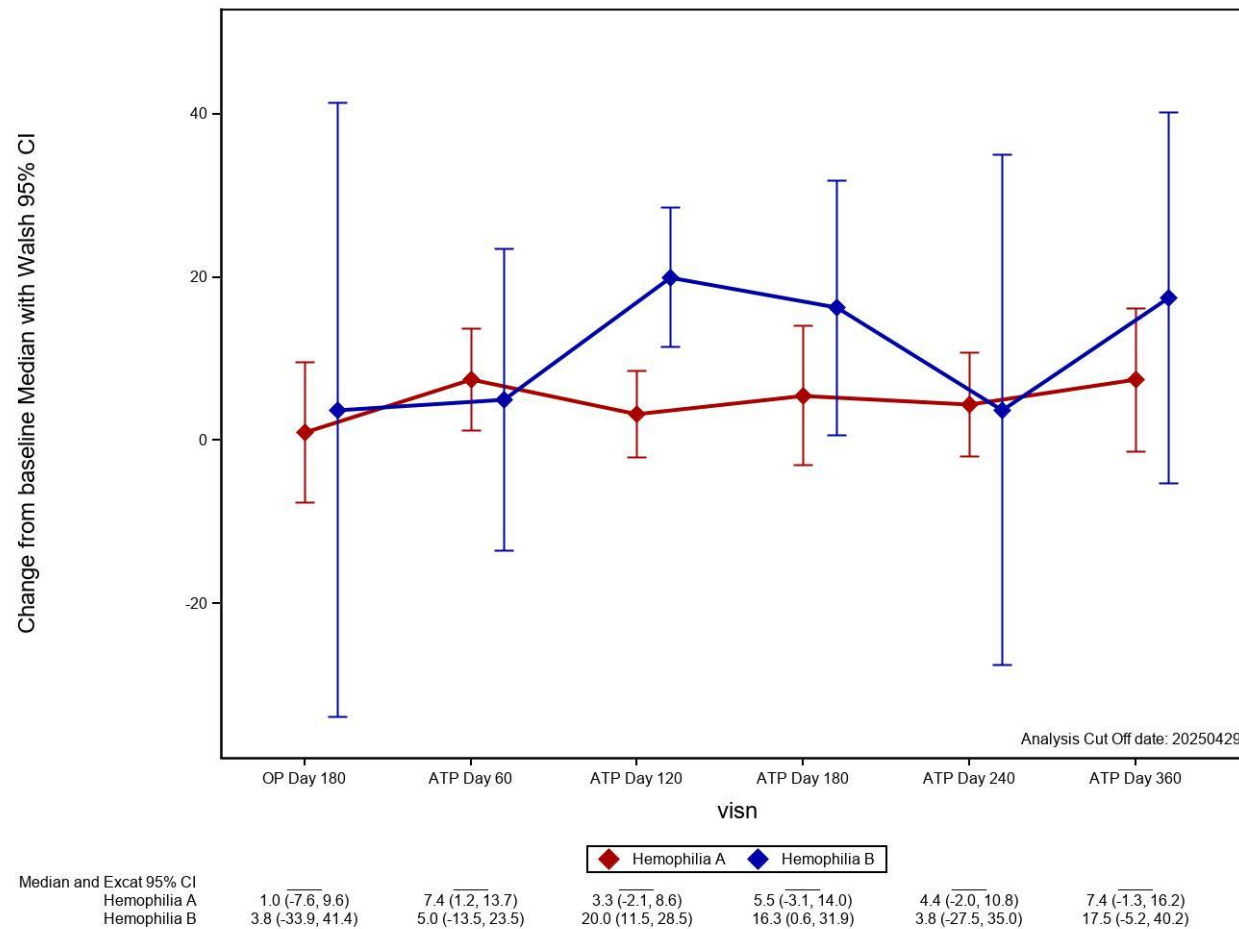
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.26.1: Hemophilia Activities List for Adults (HAL) - Lying / sitting / kneeling / standing-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



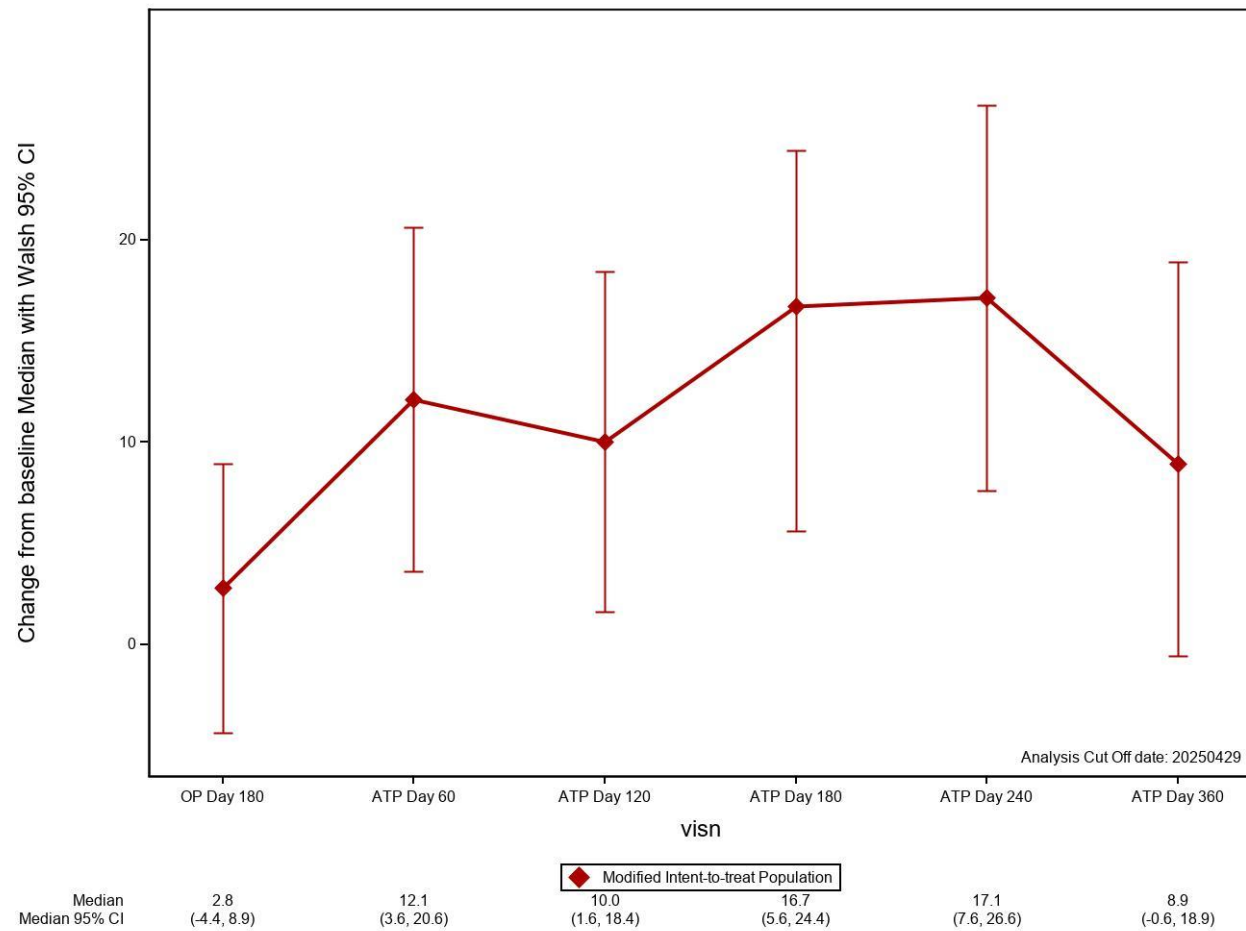
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.26.2: Hemophilia Activities List for Adults (HAL) - Lying / sitting / kneeling / standing-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



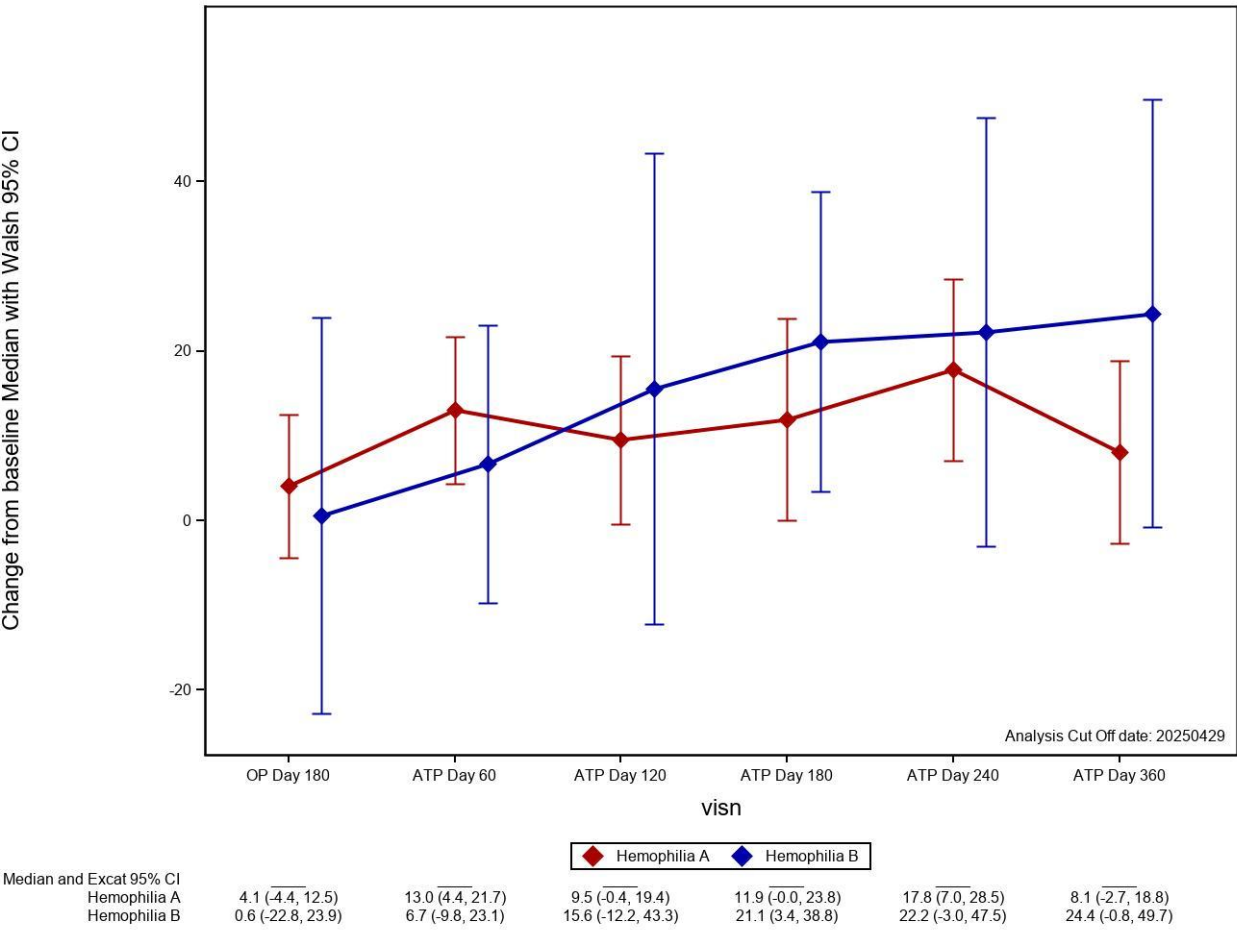
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.27.1: Hemophilia Activities List for Adults (HAL) - Functions of the legs- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



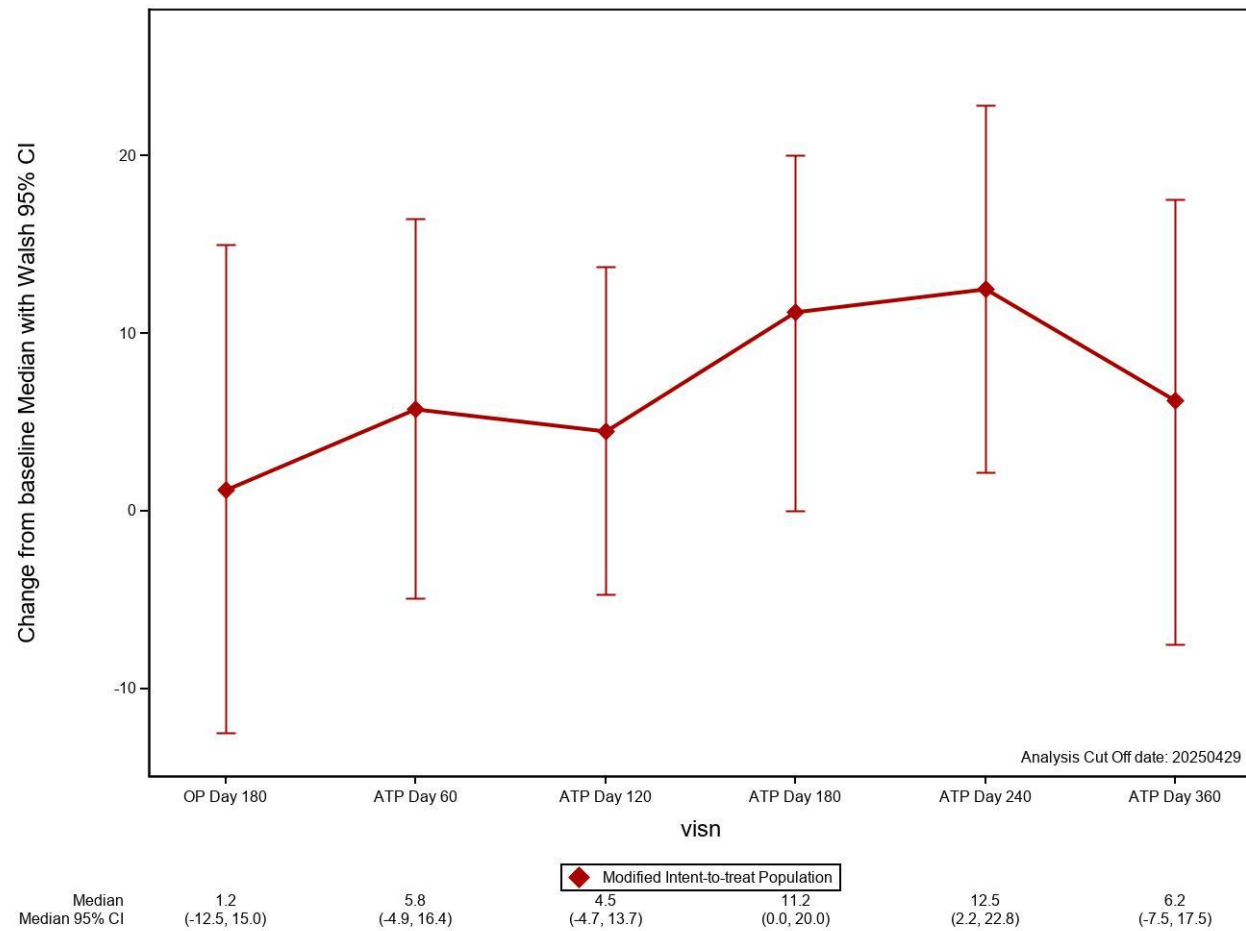
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.27.2: Hemophilia Activities List for Adults (HAL) - Functions of the legs-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



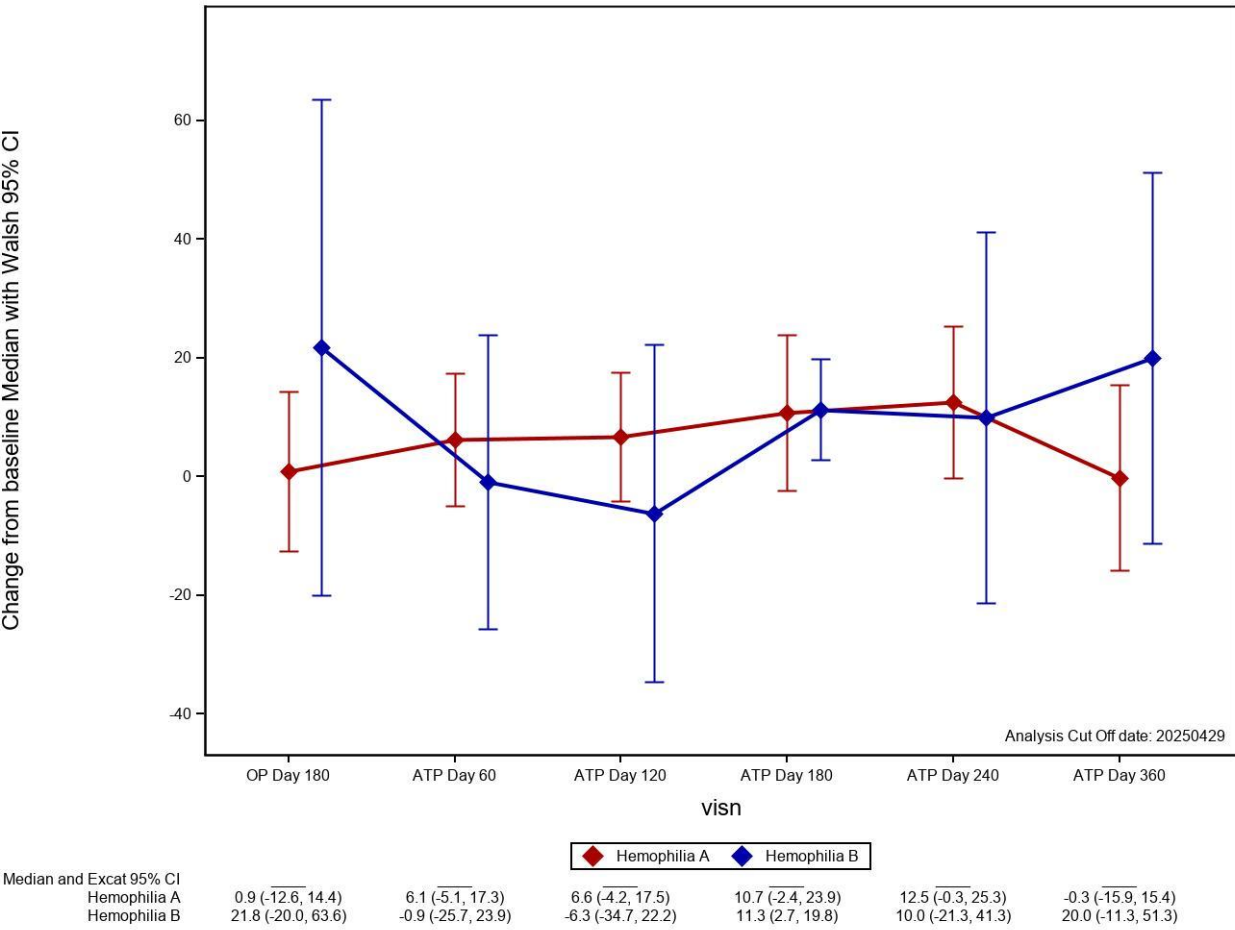
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.28.1: Hemophilia Activities List for Adults (HAL) - Functions of the arms-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



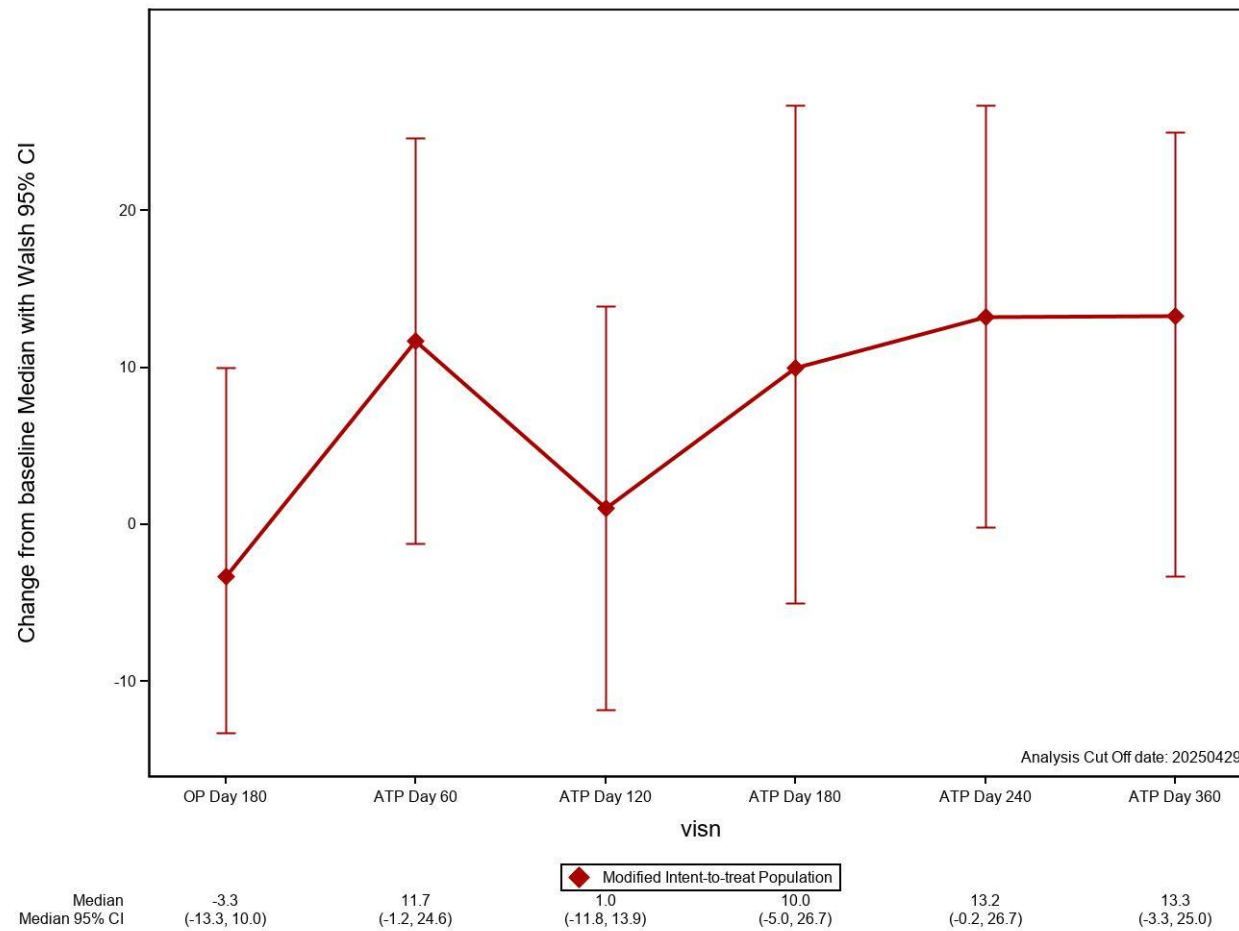
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.28.2: Hemophilia Activities List for Adults (HAL) - Functions of the arms-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



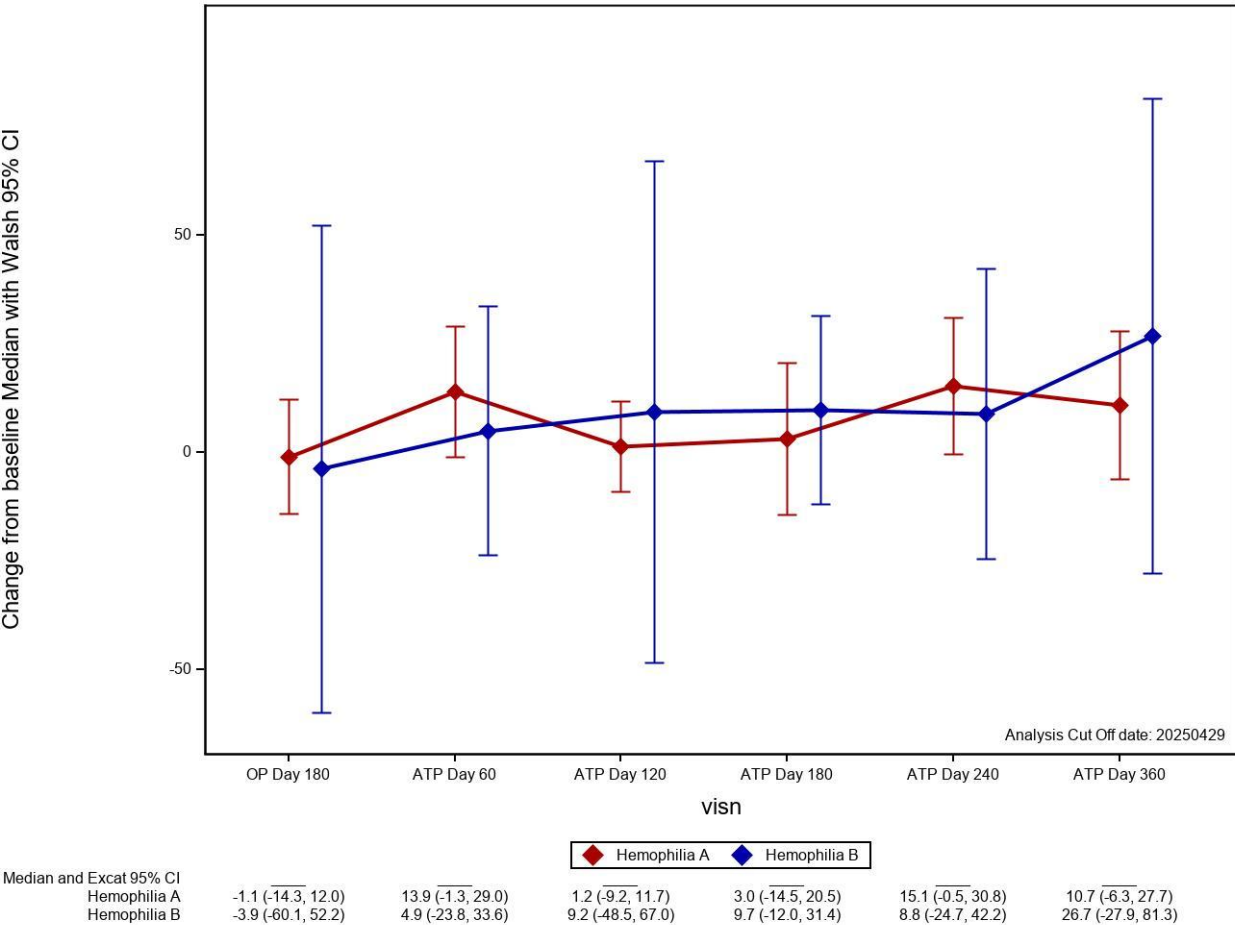
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.29.1: Hemophilia Activities List for Adults (HAL) - Use of transportation-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



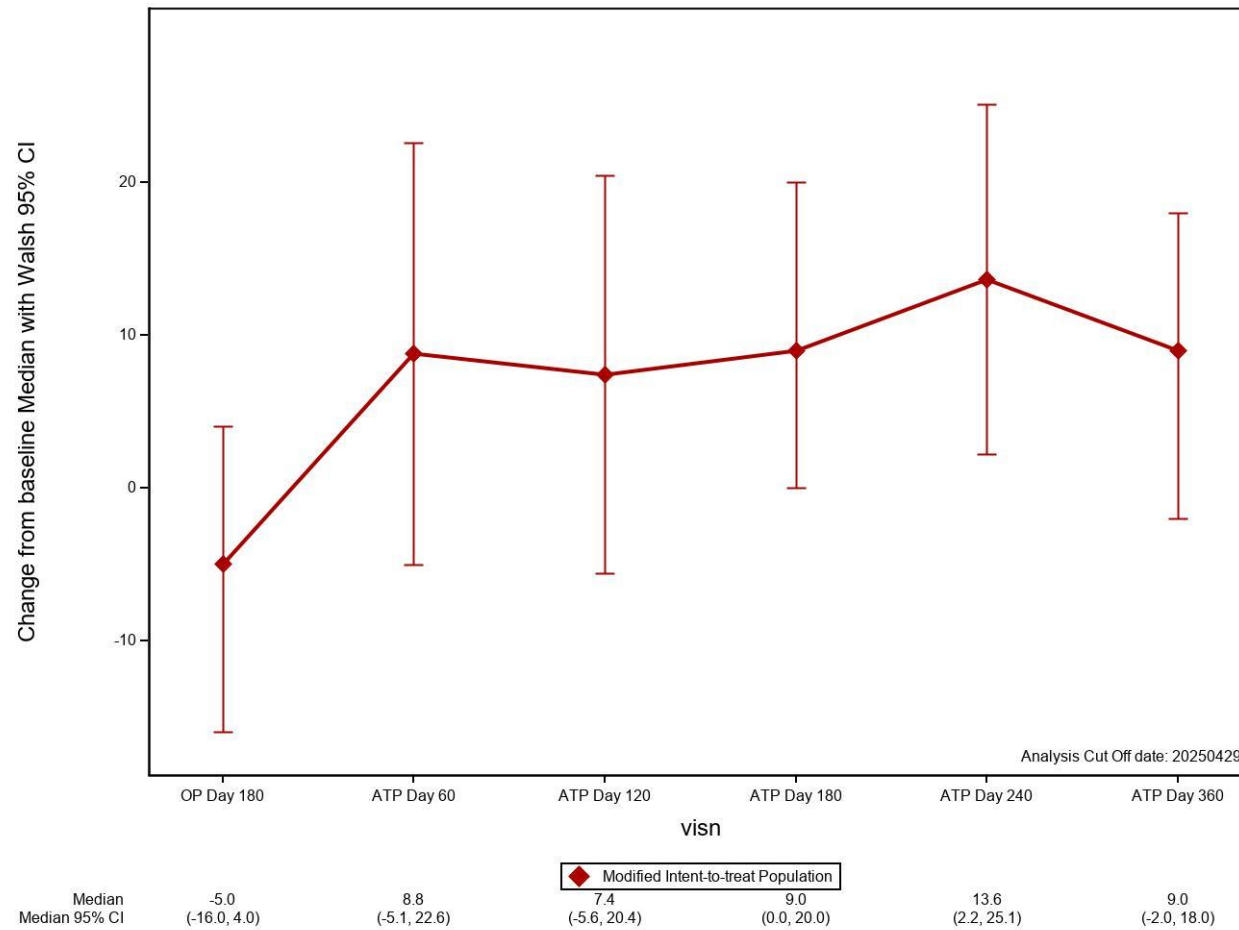
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.29.2: Hemophilia Activities List for Adults (HAL) - Use of transportation-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



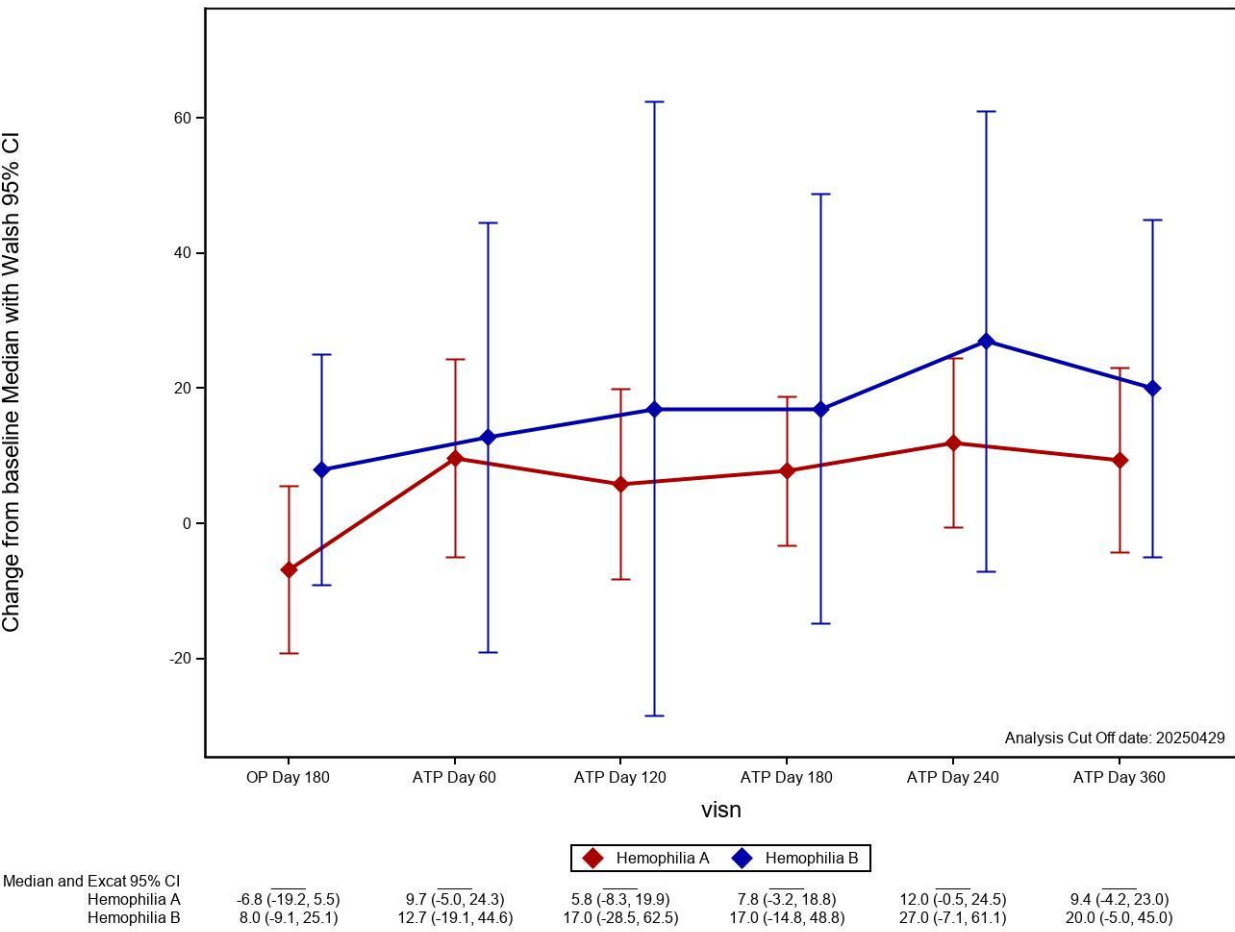
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.30.1: Hemophilia Activities List for Adults (HAL) - Self care- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



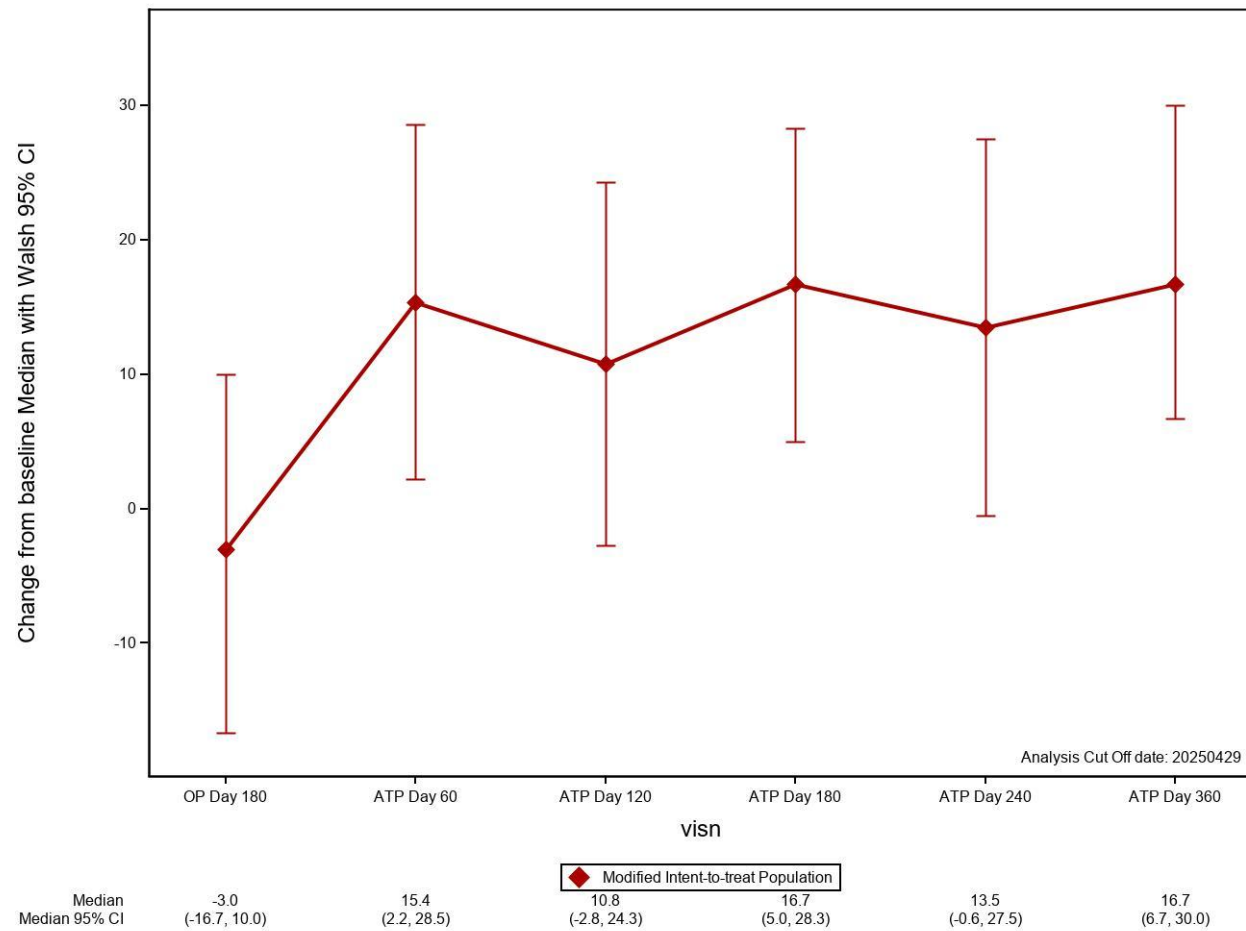
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.30.2: Hemophilia Activities List for Adults (HAL) - Self care-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



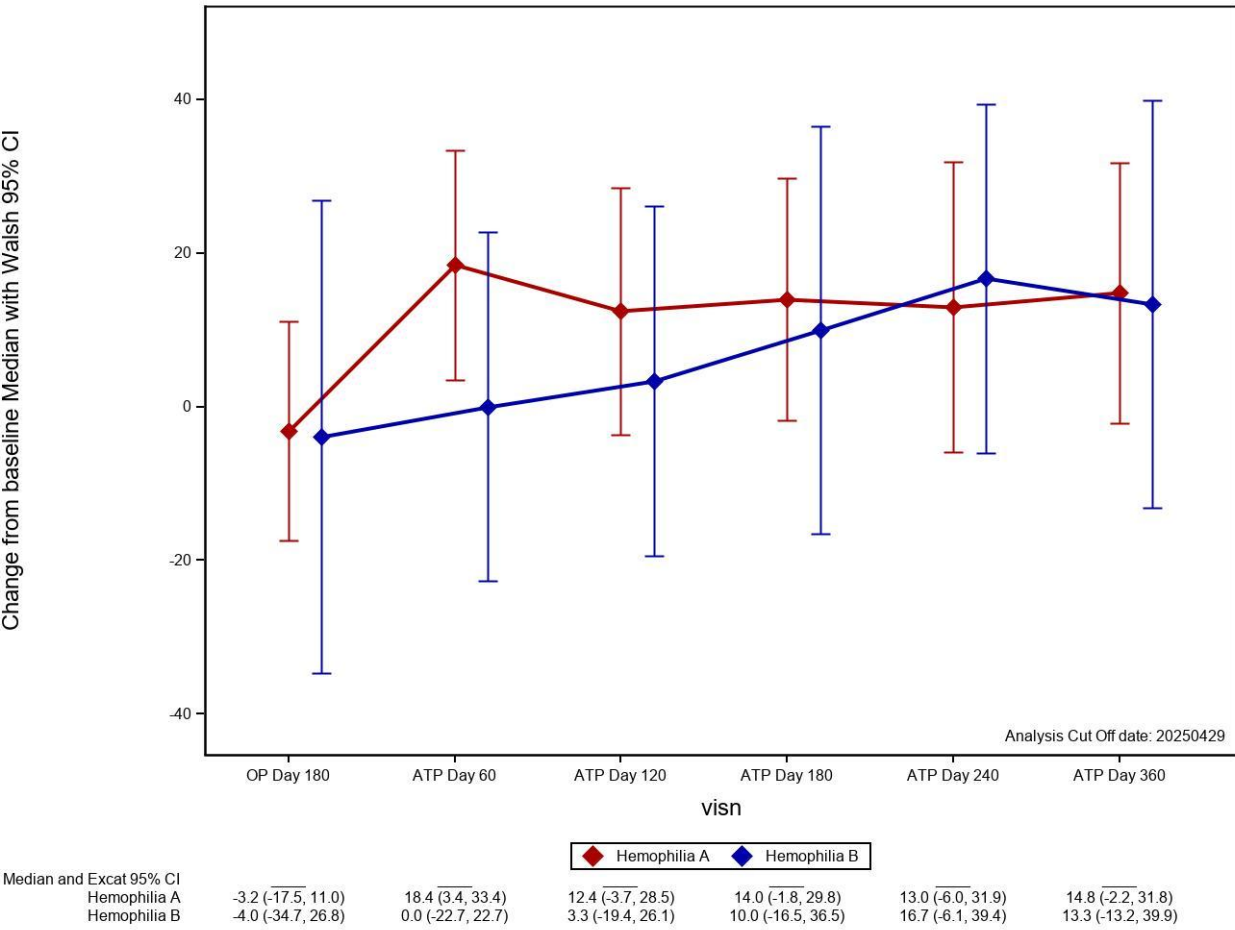
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.31.1: Hemophilia Activities List for Adults (HAL) - Household tasks-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



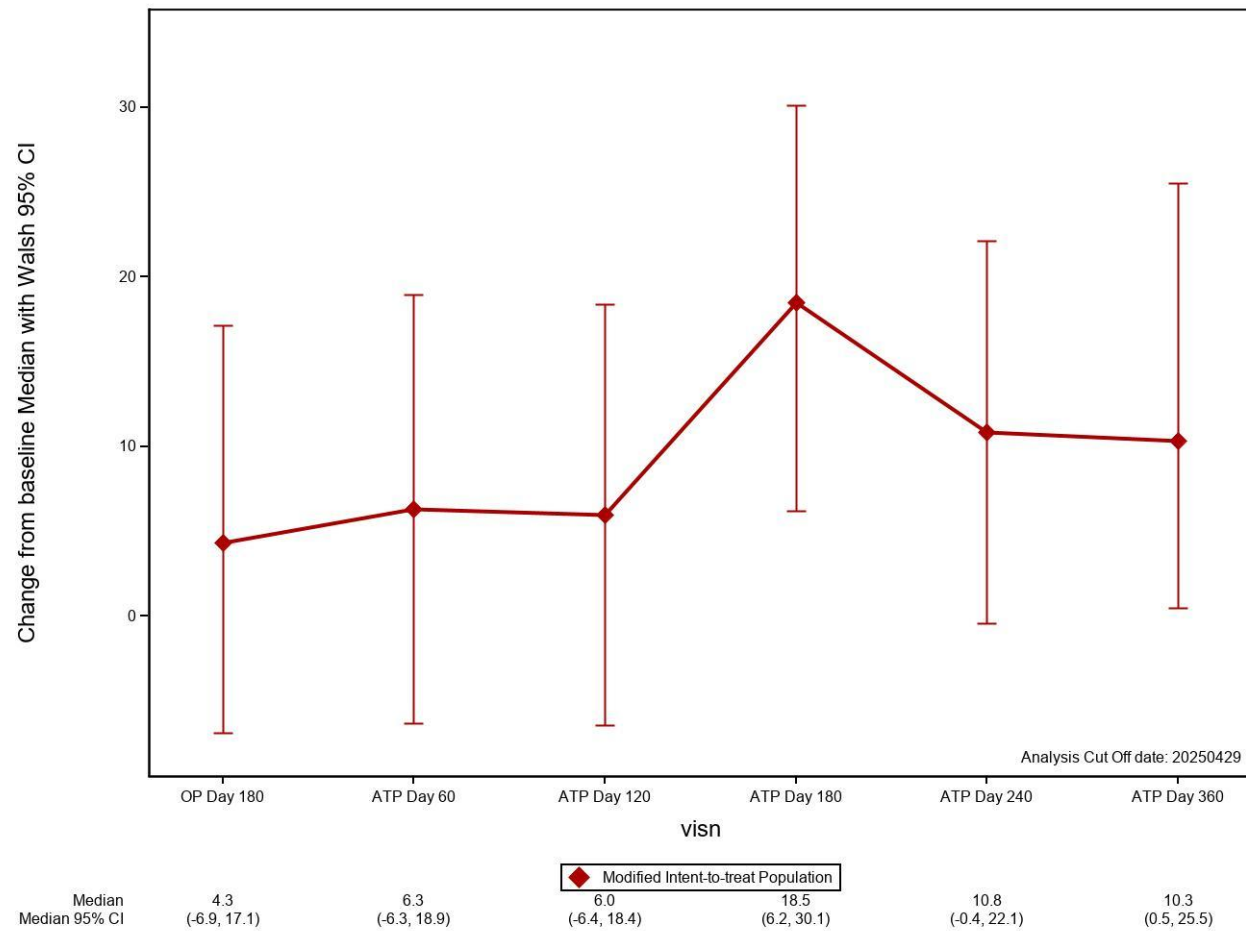
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.31.2: Hemophilia Activities List for Adults (HAL) - Household tasks-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



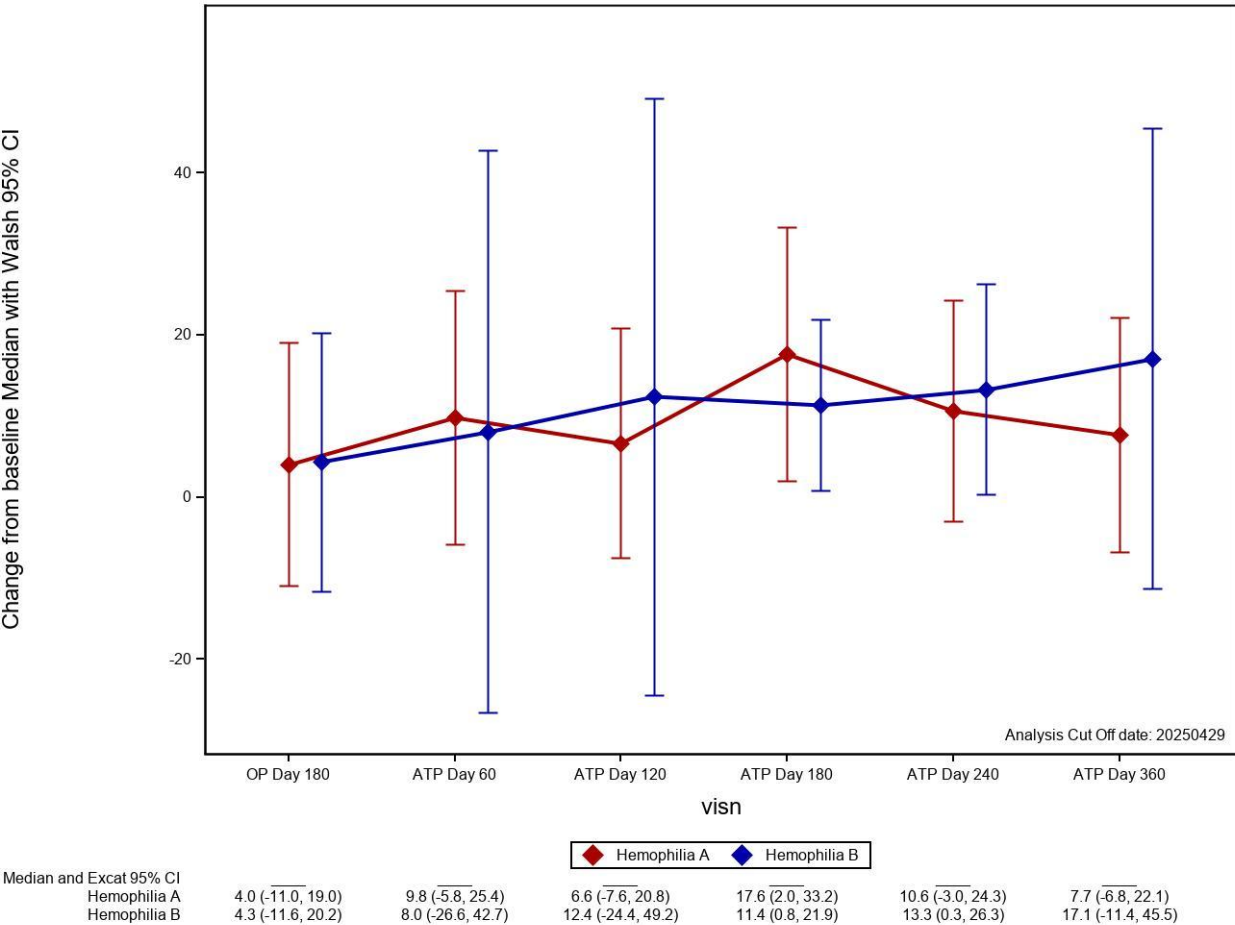
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.32.1: Hemophilia Activities List for Adults (HAL) - Leisure activities and sports- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



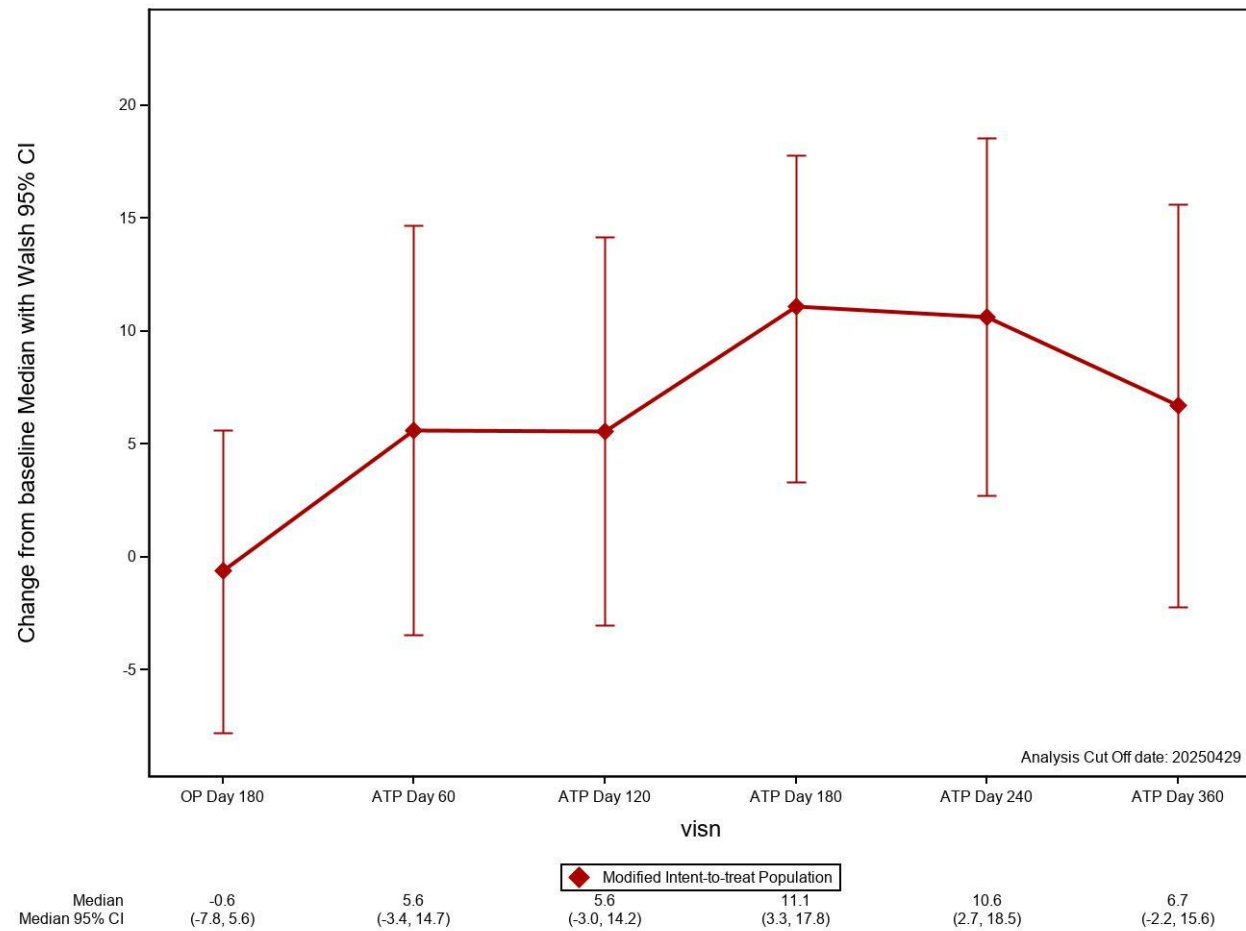
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.32.2: Hemophilia Activities List for Adults (HAL) - Leisure activities and sports- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



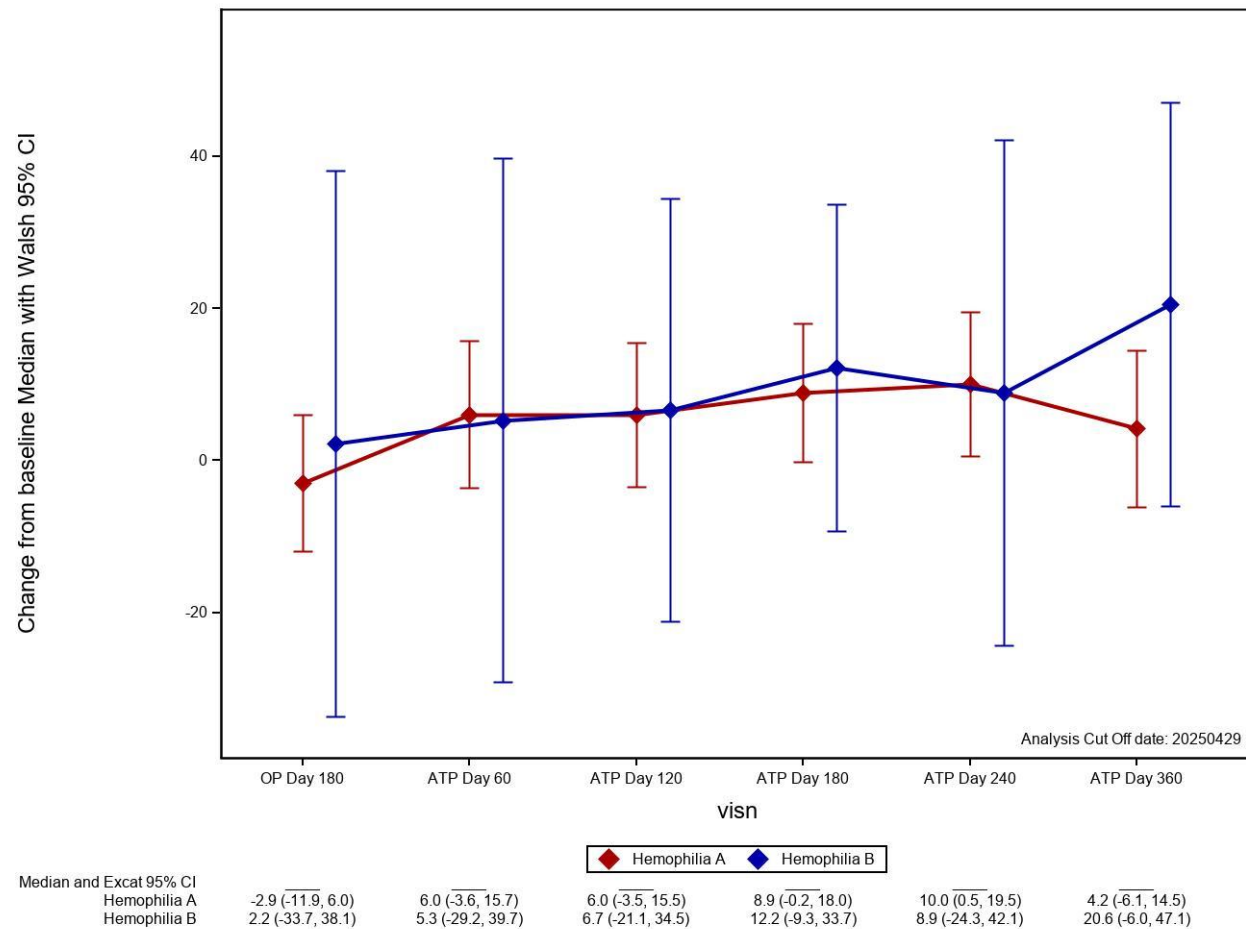
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.33.1: Hemophilia Activities List for Adults (HAL) - Upper Extremity Activities- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



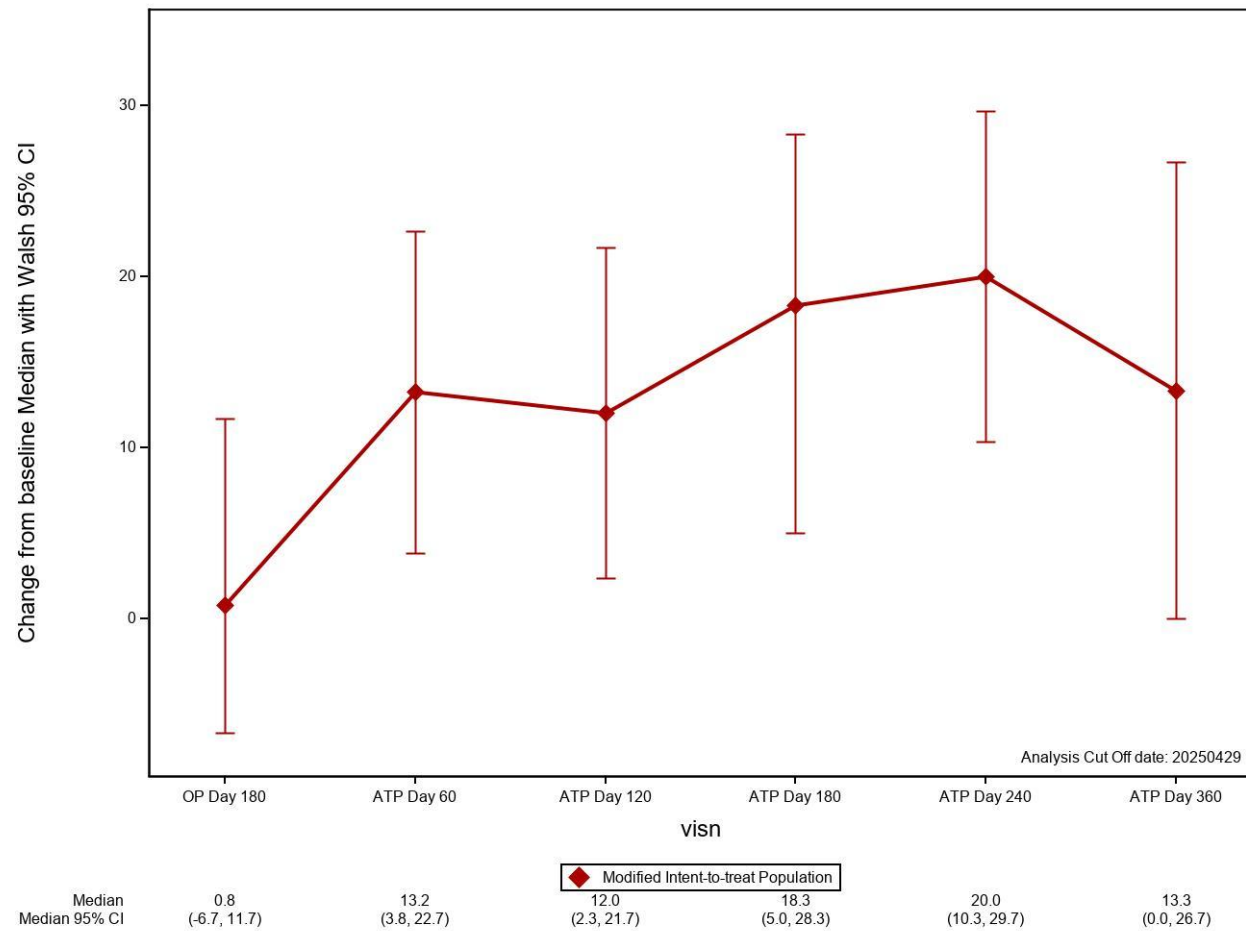
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.33.2: Hemophilia Activities List for Adults (HAL) - Upper Extremity Activities- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



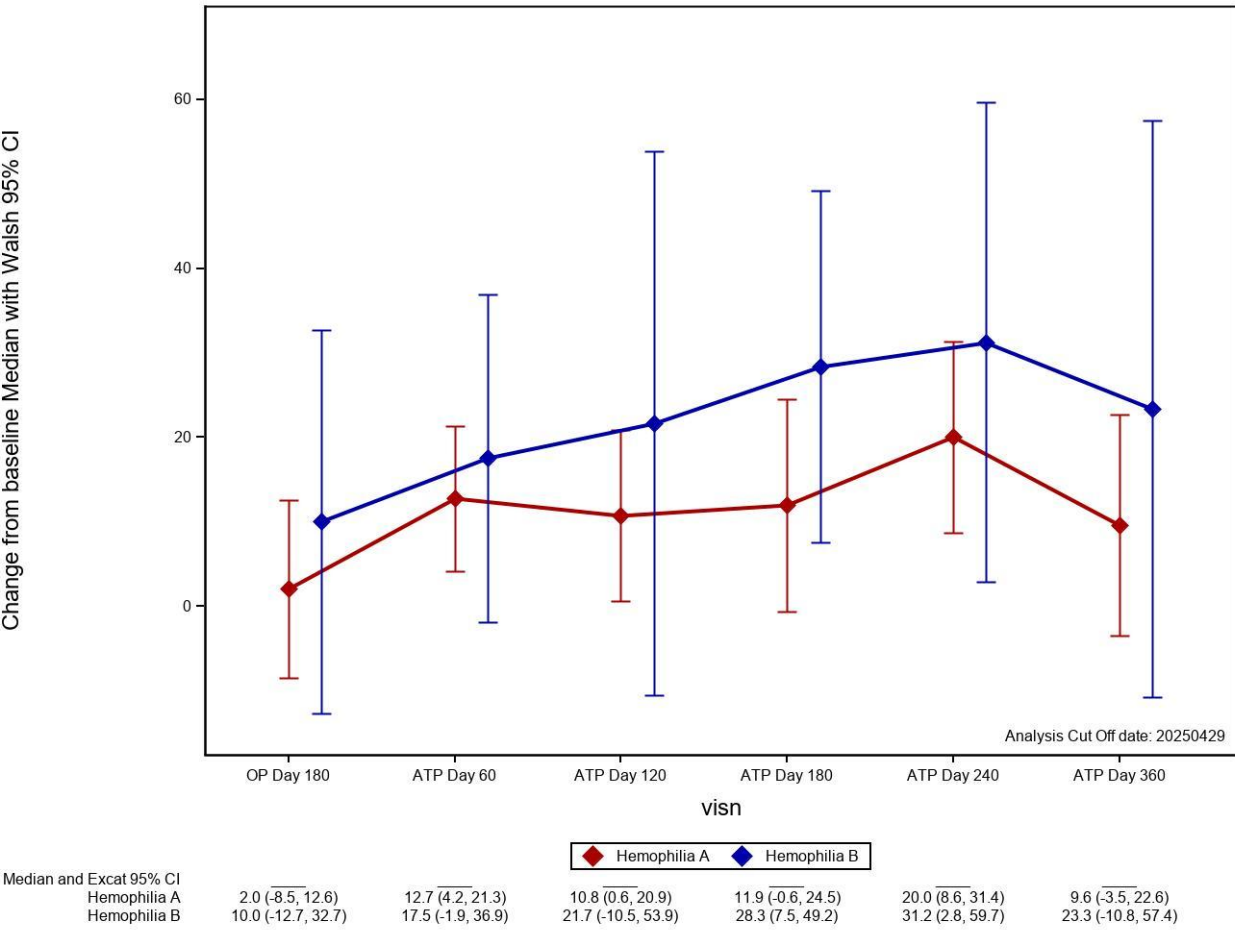
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.34.1: Hemophilia Activities List for Adults (HAL) - Basic Lower Extremity Activities- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



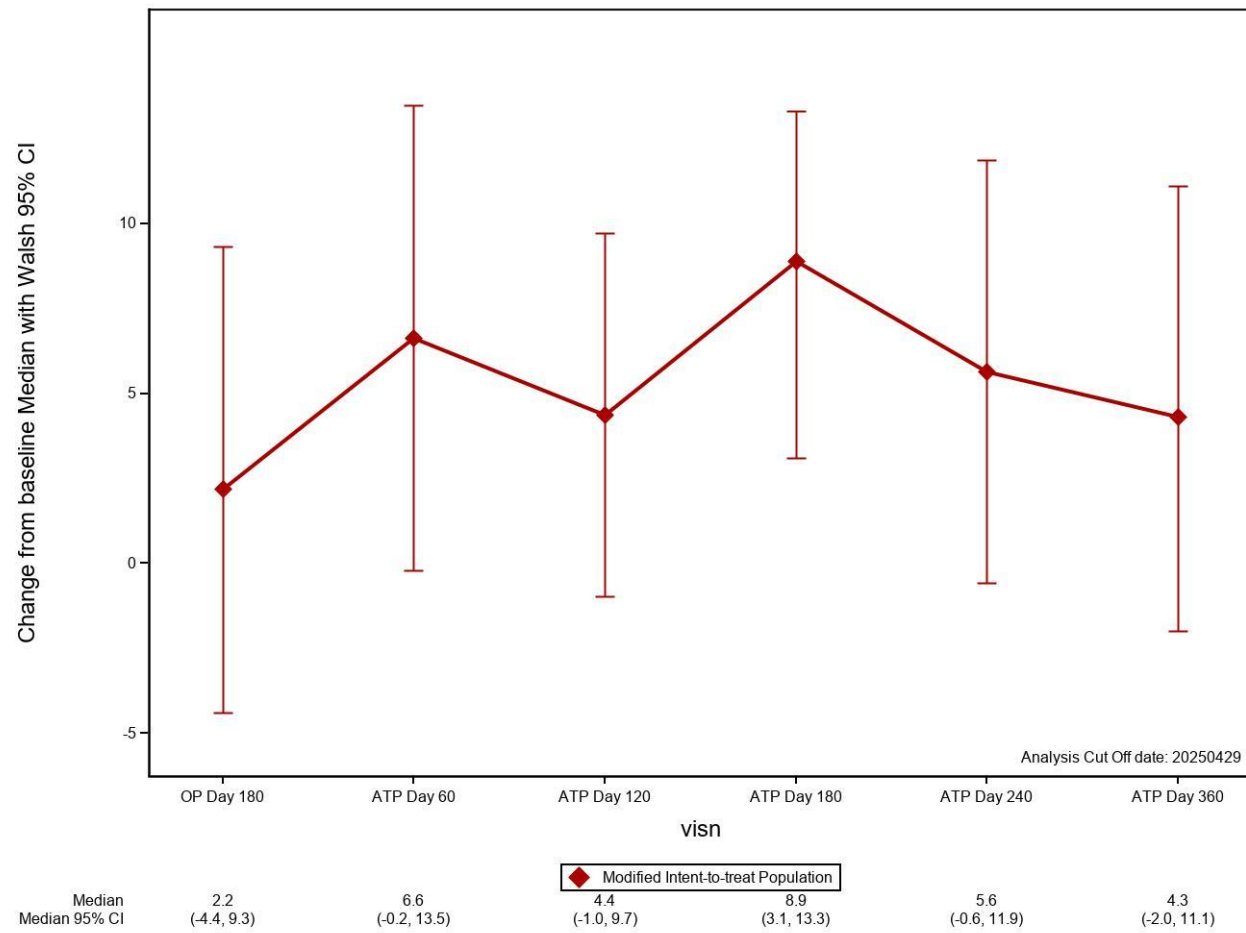
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.34.2: Hemophilia Activities List for Adults (HAL) - Basic Lower Extremity Activities- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



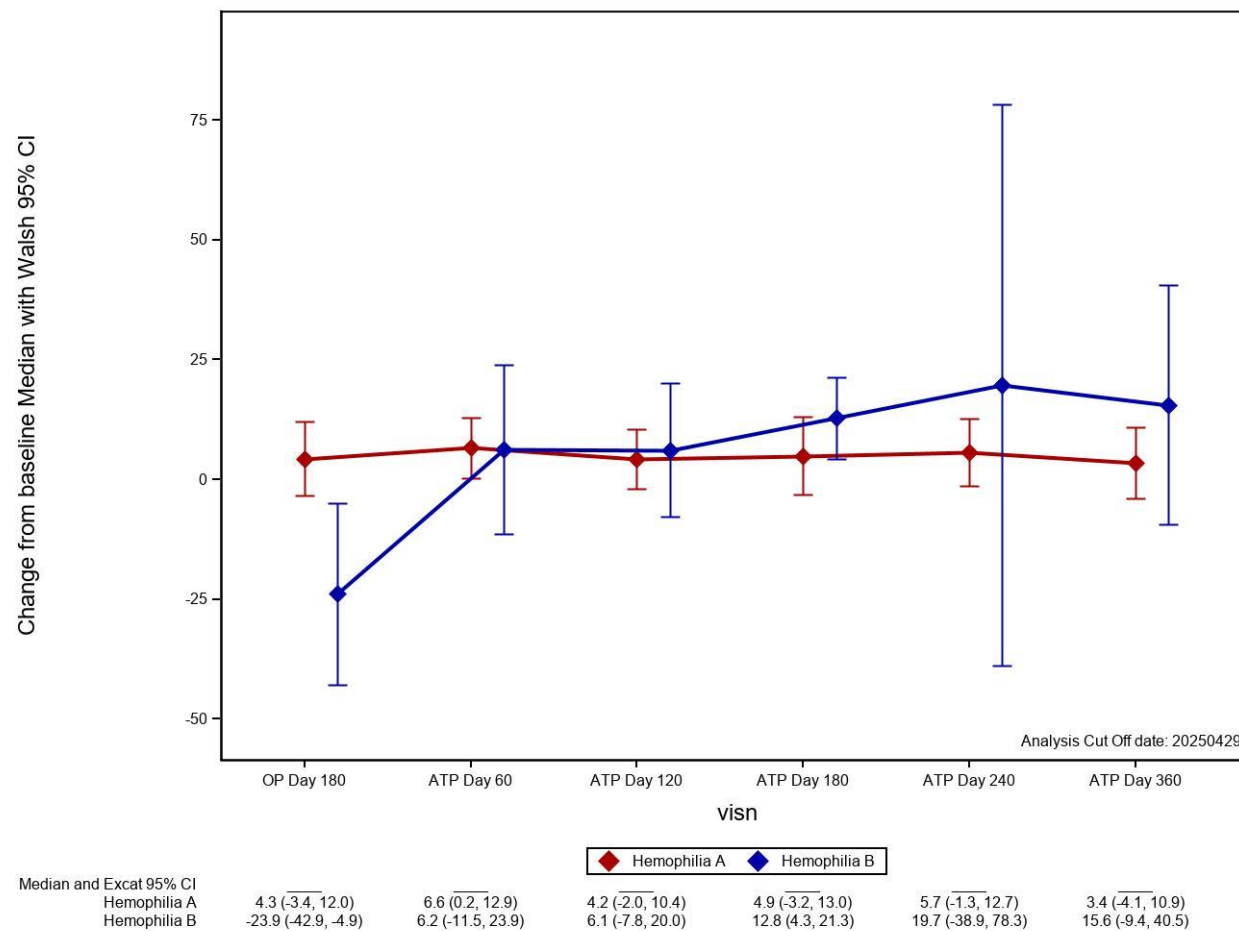
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.35.1: Hemophilia Activities List for Adults (HAL) - Complex Lower Extremity Activities-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



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

FIGURE .6.2.35.2: Hemophilia Activities List for Adults (HAL) - Complex Lower Extremity Activities-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



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

6.2 Ped-HAL

6.2.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.1.6. Ped-HAL Global score response (≥ 15 points Improvement from baseline) analysis.

Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Overall								
-	13	2.2 (16.9%)	6.7 (51.5%)	7.6 (58.5%)	3.06 (0.62, 15.04) 0.1682	5.30 (0.54, 52.14) 0.1525	34.6 (-8.8, 78.1) 0.1178	
Disease type								
Hemophilia A	10	2.0 (20.0%)	4.4 (44.0%)	5.3 (53.0%)	2.19 (0.44, 10.81) 0.3372	3.14 (0.33, 30.16) 0.3222	24.0 (-21.1, 69.1) 0.2970	
Hemophilia B	3	0.2 (6.7%)	2.3 (76.7%)	2.3 (76.7%)	1.74 (0.22, 13.89) 0.5998	3.03 (0.08, 118.53) 0.5517	26.7 (-55.9, 109.2) 0.5245	0.9875
Age group								
Adults (Ages 18 to < 75)	1	1.0 (100.0%)	0.4 (40.0%)	0.3 (30.0%)	-	-	-	
Adolescents (Ages 12 to < 18)	12	1.2 (10.0%)	6.3 (52.5%)	7.3 (60.8%)	-	-	-	-
Region								
Asia	6	1.0 (16.7%)	2.4 (40.0%)	2.3 (38.3%)	-	-	-	
Europe	3	1.0 (33.3%)	1.0 (33.3%)	2.0 (66.7%)	-	-	-	
Other	4	0.2 (5.0%)	3.3 (82.5%)	3.3 (82.5%)	-	-	-	-
White Race								
White	5	1.0 (20.0%)	2.7 (54.0%)	4.0 (80.0%)	-	-	-	
Other	8	1.2 (15.0%)	4.0 (50.0%)	3.6 (45.0%)	-	-	-	-

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visits data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD were performed by imputation, and the results were pooled using Rubin's rules.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 7.

Incidences by phase					ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months				OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Lying / sitting / kneeling / standing- (Normalized)								
Overall								
Modified Intent-to-Treat	13	1 (7.7%)	5 (38.5%)	6 (46.2%)	5.00 (0.87, 28.86) 0.0720	7.50 (1.08, 51.95) 0.0413	30.8 (5.7, 55.9) 0.0162	
Set								
Disease type								
Hemophilia A	10	1 (10.0%)	4 (40.0%)	4 (40.0%)	4.00 (0.73, 21.84) 0.1094	6.00 (0.87, 41.44) 0.0692	30.0 (1.6, 58.4) 0.0384	
Hemophilia B	3	0 (0.0%)	1 (33.3%)	2 (66.7%)	2.00 (0.50, 8.00) 0.3270	4.00 (0.36, 44.11) 0.2577	33.3 (-20.0, 86.7) 0.2207	0.7974
Region								
Asia	6	0 (0.0%)	2 (33.3%)	1 (16.7%)	-	-	-	
Europe	3	1 (33.3%)	2 (66.7%)	2 (66.7%)	-	-	-	
Other	4	0 (0.0%)	1 (25.0%)	3 (75.0%)	-	-	-	-
White Race								
White	5	1 (20.0%)	2 (40.0%)	4 (80.0%)	-	-	-	
Other	8	0 (0.0%)	3 (37.5%)	2 (25.0%)	-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 2 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Functions of the legs-(Normalized)										
Overall										
Modified Intent-to-Treat Set	13	4 (30.8%)	4 (30.8%)	6 (46.2%)	1.00 (0.30, 3.32) 1.0000		1.00 (0.18, 5.66) 1.0000		-0.0 (-36.9, 36.9) 1.0000	
Disease type										
Hemophilia A	10	4 (40.0%)	4 (40.0%)	4 (40.0%)	1.00 (0.30, 3.32) 1.0000		1.00 (0.14, 7.39) 1.0000		0.0 (-48.0, 48.0) 1.0000	
Hemophilia B	3	0 (0.0%)	0 (0.0%)	2 (66.7%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	
Region										
Asia	6	2 (33.3%)	3 (50.0%)	1 (16.7%)	-		-		-	
Europe	3	2 (66.7%)	0 (0.0%)	2 (66.7%)	-		-		-	
Other	4	0 (0.0%)	1 (25.0%)	3 (75.0%)	-		-		-	
White Race										
White	5	2 (40.0%)	1 (20.0%)	4 (80.0%)	-		-		-	
Other	8	2 (25.0%)	3 (37.5%)	2 (25.0%)	-		-		-	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 3 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months				OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Functions of the arms-(Normalized)								
Overall								
Set Modified Intent-to-Treat	13	2 (15.4%)	5 (38.5%)	8 (61.5%)	2.50 (0.49, 12.89) 0.2734	3.44 (0.40, 29.72) 0.2619	23.1 (-14.8, 60.9) 0.2323	
Disease type								
Hemophilia A	10	2 (20.0%)	4 (40.0%)	6 (60.0%)	2.00 (0.37, 10.92) 0.4235	2.67 (0.25, 28.44) 0.4167	20.0 (-26.4, 66.4) 0.3980	
Hemophilia B	3	0 (0.0%)	1 (33.3%)	2 (66.7%)	2.00 (0.50, 8.00) 0.3270	4.00 (0.36, 44.11) 0.2577	33.3 (-20.0, 86.7) 0.2207	0.8147
Region								
Asia	6	1 (16.7%)	2 (33.3%)	2 (33.3%)	-	-	-	
Europe	3	1 (33.3%)	1 (33.3%)	3 (100.0%)	-	-	-	
Other	4	0 (0.0%)	2 (50.0%)	3 (75.0%)	-	-	-	-
White Race								
White	5	1 (20.0%)	2 (40.0%)	5 (100.0%)	-	-	-	
Other	8	1 (12.5%)	3 (37.5%)	3 (37.5%)	-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 4 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Use of transportation-(Normalized)										
Overall										
Modified Intent-to-Treat Set	13	4 (30.8%)	3 (23.1%)	5 (38.5%)	0.75 (0.21, 2.66) 0.6558		0.68 (0.12, 3.77) 0.6543		-7.7 (-41.1, 25.8) 0.6522	
Disease type										
Hemophilia A	10	4 (40.0%)	3 (30.0%)	4 (40.0%)	0.75 (0.21, 2.66) 0.6558		0.64 (0.09, 4.44) 0.6541		-10.0 (-53.4, 33.4) 0.6514	
Hemophilia B	3	0 (0.0%)	0 (0.0%)	1 (33.3%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	0.8512
Region										
Asia	6	2 (33.3%)	2 (33.3%)	2 (33.3%)	-		-		-	
Europe	3	2 (66.7%)	0 (0.0%)	1 (33.3%)	-		-		-	
Other	4	0 (0.0%)	1 (25.0%)	2 (50.0%)	-		-		-	-
White Race										
White	5	2 (40.0%)	1 (20.0%)	3 (60.0%)	-		-		-	
Other	8	2 (25.0%)	2 (25.0%)	2 (25.0%)	-		-		-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 5 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Self care-(Normalized)										
Overall										
Modified Intent-to-Treat Set	13	3 (23.1%)	3 (23.1%)	6 (46.2%)	1.00 (0.20, 4.95) 1.0000		1.00 (0.12, 8.01) 1.0000		0.0 (-36.9, 36.9) 1.0000	
Disease type										
Hemophilia A	10	3 (30.0%)	3 (30.0%)	4 (40.0%)	1.00 (0.20, 4.95) 1.0000		1.00 (0.10, 9.84) 1.0000		0.0 (-48.0, 48.0) 1.0000	
Hemophilia B	3	0 (0.0%)	0 (0.0%)	2 (66.7%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	
Region										
Asia	6	1 (16.7%)	2 (33.3%)	2 (33.3%)	-		-		-	
Europe	3	2 (66.7%)	0 (0.0%)	2 (66.7%)	-		-		-	
Other	4	0 (0.0%)	1 (25.0%)	2 (50.0%)	-		-		-	
White Race										
White	5	2 (40.0%)	0 (0.0%)	3 (60.0%)	-		-		-	
Other	8	1 (12.5%)	3 (37.5%)	3 (37.5%)	-		-		-	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 6 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months		RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)					
DOMAIN: Household tasks- (Normalized)									
Overall									
Set Modified Intent-to-Treat	13	3 (23.1%)	4 (30.8%)	6 (46.2%)		1.33 (0.50, 3.55) 0.5650	1.48 (0.39, 5.58) 0.5611	7.7 (-18.1, 33.5) 0.5586	
Disease type									
Hemophilia A	10	2 (20.0%)	3 (30.0%)	5 (50.0%)		1.50 (0.38, 6.00) 0.5664	1.71 (0.28, 10.59) 0.5618	10.0 (-23.4, 43.4) 0.5571	
Hemophilia B	3	1 (33.3%)	1 (33.3%)	1 (33.3%)		1.00 (1.00, 1.00) .	1.00 (., .) .	-0.0 (-0.0, -0.0) .	0.5706
Region									
Asia	6	1 (16.7%)	2 (33.3%)	2 (33.3%)		-	-	-	
Europe	3	2 (66.7%)	1 (33.3%)	2 (66.7%)		-	-	-	
Other	4	0 (0.0%)	1 (25.0%)	2 (50.0%)		-	-	-	-
White Race									
White	5	2 (40.0%)	1 (20.0%)	3 (60.0%)		-	-	-	
Other	8	1 (12.5%)	3 (37.5%)	3 (37.5%)		-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.2.4. Ped-HAL response (≥ 15 points Improvement from baseline) analysis

Modified Intent-to-Treat Set

Table page 7 of 7.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)				OR Interacti on p- value [2]	
					RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		
DOMAIN: Leisure activities and sports-(Normalized)									
Overall									
Set Modified Intent-to-Treat	11	2 (18.2%)	1 (9.1%)	3 (27.3%)	0.50 (0.05, 5.51) 0.5714	0.45 (0.03, 6.97) 0.5680	-9.1 (-39.5, 21.3) 0.5577		
Disease type									
Hemophilia A	9	2 (22.2%)	1 (11.1%)	3 (33.3%)	0.50 (0.05, 5.51) 0.5714	0.44 (0.03, 7.44) 0.5674	-11.1 (-48.1, 25.9) 0.5563		
Region									
Asia	5	0 (0.0%)	1 (20.0%)	1 (20.0%)	-	-	-		
Europe	3	2 (66.7%)	0 (0.0%)	1 (33.3%)	-	-	-		
Other	3	0 (0.0%)	0 (0.0%)	1 (33.3%)	-	-	-		-
White Race									
White	5	2 (40.0%)	0 (0.0%)	1 (20.0%)	-	-	-		
Other	6	0 (0.0%)	1 (16.7%)	2 (33.3%)	-	-	-		-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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6.2.2 Sensitivitätsanalyse

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 Datacut: B7841005 (29APR2025)
 TABLE 6.1.36. Pediatric Hemophilia Activities List (pedHAL) - Total Score-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=12 63.8 (21.32) 51.1 61.00 80.2	-	-
OP Day 180	n=12 60.7 (19.38) 47.7 50.49 72.8	n=12 -3.1 (20.42) -19.8 -3.58 10.2	-4.4 (-20.9, 12.1)
ATP Baseline	n=11 54.8 (20.94) 40.0 51.32 67.5	-	-
ATP Day 180	n=11 70.7 (27.81) 55.8 75.09 96.6	n=9 11.9 (15.01) 6.4 9.06 16.5	14.4 (1.1, 27.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	20.6 (-2.2, 43.3)
ATP Day 360	n=11 76.8 (26.17) 63.5 78.57 98.9	n=10 20.3 (15.95) 8.8 21.16 31.3	0.0760 20.1 (6.0, 34.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
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 TABLE 6.1.36. Pediatric Hemophilia Activities List (pedHAL) - Total Score-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 61.9 (22.79) 49.8 58.74 79.5	-	-
OP Day 180	n=10 63.3 (20.26) 47.9 57.10 82.3	n=10 1.4 (19.18) -11.3 -0.81 14.4	0.3 (-14.9, 15.6)
ATP Baseline	n=9 53.5 (22.52) 40.0 51.32 61.1	-	-
ATP Day 180	n=9 69.1 (28.80) 56.5 75.09 91.7	n=8 12.6 (15.89) 2.3 11.89 21.2	9.4 (-3.1, 22.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	10.0 (-17.9, 37.9)
ATP Day 360	n=9 74.0 (27.92) 63.5 77.02 98.1	n=8 18.2 (17.37) 4.8 12.11 34.2	0.4435 14.2 (0.6, 27.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.36. Pediatric Hemophilia Activities List (pedHAL) - Total Score-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=2 73.2 (10.74) 65.6 73.18 80.8	-	-
OP Day 180	n=2 47.4 (3.94) 44.6 47.40 50.2	n=2 -25.8 (6.80) -30.6 -25.77 -21.0	-28.0 (-33.5, -22.6)
ATP Baseline	n=2 60.6 (15.74) 49.4 60.57 71.7	-	-
ATP Day 180	n=2 77.9 (31.22) 55.8 77.92 100.0	n=1 6.4 (-) 6.4 6.42 6.4	23.0 (10.2, 35.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	51.1 (33.0, 69.2) <.0001
ATP Day 360	n=2 89.3 (15.15) 78.6 89.29 100.0	n=2 28.7 (0.59) 28.3 28.72 29.1	28.5 (28.0, 29.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 6.1.37. Pediatric Hemophilia Activities List (pedHAL) - Lying / sitting / kneeling / standing- (Normalized).

Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=11 59.3 (22.00) 46.0 60.00 76.0	-	-
OP Day 180	n=12 60.5 (21.05) 44.0 58.00 78.0	n=11 3.1 (16.03) -12.0 4.00 10.0	1.5 (-7.0, 14.0)
ATP Baseline	n=12 57.2 (26.36) 44.0 53.00 73.0	-	-
ATP Day 180	n=11 72.4 (27.30) 58.0 76.00 100.0	n=10 14.6 (12.65) 2.0 14.00 20.0	14.5 (-4.0, 29.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	6.5 (-6.0, 18.0)
ATP Day 360	n=11 78.5 (25.08) 64.0 86.00 100.0	n=11 21.3 (16.91) 10.0 20.00 40.0	0.3311 19.5 (12.0, 36.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 6.1.37. Pediatric Hemophilia Activities List (pedHAL) - Lying / sitting / kneeling / standing- (Normalized)

Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=9 57.8 (23.80) 46.0 60.00 70.0	-	-
OP Day 180	n=10 62.4 (22.35) 46.0 60.00 82.0	n=9 7.1 (14.87) 0.0 4.00 10.0	8.0 (-6.8, 22.8)
ATP Baseline	n=10 55.8 (27.93) 40.0 53.00 66.0	-	-
ATP Day 180	n=9 69.8 (28.75) 58.0 76.00 88.0	n=9 14.0 (13.27) 2.0 12.00 18.0	15.8 (4.1, 27.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	3.2 (-9.0, 15.5)
ATP Day 360	n=9 76.9 (26.83) 64.0 86.00 98.0	n=9 21.1 (18.87) 10.0 14.00 40.0	0.5704 27.3 (9.9, 44.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 6.1.37. Pediatric Hemophilia Activities List (pedHAL) - Lying / sitting / kneeling / standing- (Normalized)

Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=2 66.0 (14.14) 56.0 66.00 76.0	-	-
OP Day 180	n=2 51.0 (12.73) 42.0 51.00 60.0	n=2 -15.0 (1.41) -16.0 -15.00 -14.0	-15.5 (-16.6, -14.3)
ATP Baseline	n=2 64.0 (22.63) 48.0 64.00 80.0	-	-
ATP Day 180	n=2 84.0 (22.63) 68.0 84.00 100.0	n=1 20.0 (-) 20.0 20.00 20.0	20.2 (19.7, 20.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	35.7 (34.1, 37.3) <.0001
ATP Day 360	n=2 86.0 (19.80) 72.0 86.00 100.0	n=2 22.0 (2.83) 20.0 22.00 24.0	21.1 (18.8, 23.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.38. Pediatric Hemophilia Activities List (pedHAL) - Functions of the legs-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=11 60.3 (22.04) 43.6 60.00 80.0	-	-
OP Day 180	n=12 57.5 (24.06) 39.1 50.91 77.3	n=11 -5.8 (27.55) -34.5 -12.73 20.0	-8.2 (-30.0, 18.2)
ATP Baseline	n=11 53.6 (21.16) 40.0 58.18 60.0	-	-
ATP Day 180	n=11 72.4 (28.13) 50.9 76.36 100.0	n=9 13.9 (14.85) 7.3 10.91 21.8	10.9 (-0.9, 23.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	20.9 (-2.7, 39.1)
ATP Day 360	n=11 76.4 (26.02) 63.6 72.73 100.0	n=10 20.9 (16.22) 7.3 20.91 36.4	0.1309 21.8 (8.2, 33.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.38. Pediatric Hemophilia Activities List (pedHAL) - Functions of the legs-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=9 58.0 (23.50) 43.6 56.36 65.5	-	-
OP Day 180	n=10 62.5 (23.07) 40.0 58.18 85.5	n=9 1.4 (25.04) -20.0 0.00 20.0	0.0 (-22.7, 22.7)
ATP Baseline	n=9 52.9 (23.54) 40.0 58.18 60.0	-	-
ATP Day 180	n=9 70.7 (29.52) 50.9 76.36 94.5	n=8 14.8 (15.65) 3.6 13.64 24.5	15.5 (0.4, 30.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	13.3 (-23.0, 49.6)
ATP Day 360	n=9 74.1 (27.74) 63.6 67.27 100.0	n=8 18.6 (16.74) 5.5 15.45 30.0	0.4336 20.2 (5.4, 34.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.38. Pediatric Hemophilia Activities List (pedHAL) - Functions of the legs-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=2 70.9 (12.86) 61.8 70.91 80.0	-	-
OP Day 180	n=2 32.7 (10.29) 25.5 32.73 40.0	n=2 -38.2 (2.57) -40.0 -38.18 -36.4	-39.0 (-41.1, -37.0)
ATP Baseline	n=2 56.4 (5.14) 52.7 56.36 60.0	-	-
ATP Day 180	n=2 80.0 (28.28) 60.0 80.00 100.0	n=1 7.3 (-) 7.3 7.27 7.3	31.9 (13.2, 50.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	70.9 (50.2, 91.7) <.0001
ATP Day 360	n=2 86.4 (19.28) 72.7 86.36 100.0	n=2 30.0 (14.14) 20.0 30.00 40.0	34.7 (23.4, 46.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.39. Pediatric Hemophilia Activities List (pedHAL) - Functions of the arms-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=12 78.9 (20.95) 61.7 86.67 95.0	-	-
OP Day 180	n=12 66.6 (19.47) 53.0 68.33 76.7	n=12 -12.3 (25.43) -33.7 -11.67 11.7	-12.7 (-31.7, 8.3)
ATP Baseline	n=12 60.8 (29.10) 45.0 66.67 76.7	-	-
ATP Day 180	n=11 74.2 (30.52) 56.7 80.00 96.7	n=10 14.0 (15.85) 0.0 13.33 23.3	15.0 (-10.0, 28.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	16.7 (-1.7, 42.0) 0.0610
ATP Day 360	n=11 81.5 (25.40) 76.7 86.67 100.0	n=11 21.8 (15.87) 13.3 20.00 26.7	22.5 (15.0, 36.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.39. Pediatric Hemophilia Activities List (pedHAL) - Functions of the arms-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 76.0 (21.93) 60.0 78.33 96.7	-	-
OP Day 180	n=10 68.9 (19.42) 56.0 68.33 80.0	n=10 -7.1 (23.65) -26.7 1.67 13.3	-6.7 (-26.6, 13.2)
ATP Baseline	n=10 59.0 (31.47) 40.0 66.67 73.3	-	-
ATP Day 180	n=9 70.7 (32.61) 56.7 70.00 96.7	n=9 13.3 (16.67) 0.0 10.00 23.3	17.1 (-3.8, 37.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	15.3 (-18.4, 49.0)
ATP Day 360	n=9 79.6 (27.56) 76.7 86.67 100.0	n=9 22.2 (17.72) 13.3 20.00 26.7	0.3348 23.4 (6.7, 40.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
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 TABLE 6.1.39. Pediatric Hemophilia Activities List (pedHAL) - Functions of the arms-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=2 93.3 (0.00) 93.3 93.33 93.3	-	-
OP Day 180	n=2 55.0 (21.21) 40.0 55.00 70.0	n=2 -38.3 (21.21) -53.3 -38.33 -23.3	NE
ATP Baseline	n=2 70.0 (14.14) 60.0 70.00 80.0	-	-
ATP Day 180	n=2 90.0 (14.14) 80.0 90.00 100.0	n=1 20.0 (-) 20.0 20.00 20.0	20.1 (19.8, 20.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	NE
ATP Day 360	n=2 90.0 (14.14) 80.0 90.00 100.0	n=2 20.0 (0.00) 20.0 20.00 20.0	20.0 (20.0, 20.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.40. Pediatric Hemophilia Activities List (pedHAL) - Use of transportation-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=11 60.6 (22.99) 40.0 60.00 80.0	-	-
OP Day 180	n=12 60.0 (26.36) 43.3 53.33 80.0	n=11 3.0 (25.19) -20.0 0.00 20.0	3.3 (-23.3, 26.7)
ATP Baseline	n=12 53.9 (29.05) 40.0 46.67 73.3	-	-
ATP Day 180	n=11 67.3 (32.04) 46.7 66.67 100.0	n=10 14.0 (20.48) 0.0 13.33 26.7	13.3 (-6.7, 36.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	6.7 (-23.3, 33.3)
ATP Day 360	n=9 77.0 (31.99) 53.3 100.00 100.0	n=9 21.5 (21.80) 0.0 20.00 40.0	0.4180 26.7 (6.7, 50.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.40. Pediatric Hemophilia Activities List (pedHAL) - Use of transportation-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 57.3 (21.36) 40.0 56.67 66.7	-	-
OP Day 180	n=10 64.7 (25.35) 46.7 56.67 100.0	n=10 7.3 (21.88) -6.7 6.67 20.0	8.3 (-16.3, 33.0)
ATP Baseline	n=10 52.7 (30.54) 40.0 46.67 66.7	-	-
ATP Day 180	n=9 68.1 (30.69) 53.3 66.67 100.0	n=9 17.0 (19.18) 0.0 13.33 26.7	20.0 (-0.0, 40.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	8.3 (-25.3, 41.9)
ATP Day 360	n=8 74.2 (32.94) 50.0 90.00 100.0	n=8 21.7 (23.30) 0.0 16.67 40.0	0.5933 23.3 (-5.3, 51.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.40. Pediatric Hemophilia Activities List (pedHAL) - Use of transportation-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=1 93.3 (-) 93.3 93.33 93.3	-	-
OP Day 180	n=2 36.7 (23.57) 20.0 36.67 53.3	n=1 -40.0 (-) -40.0 -40.00 -40.0	NE
ATP Baseline	n=2 60.0 (28.28) 40.0 60.00 80.0	-	-
ATP Day 180	n=2 63.3 (51.85) 26.7 63.33 100.0	n=1 -13.3 (-) -13.3 -13.33 -13.3	11.9 (-7.5, 31.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	NE
ATP Day 360	n=1 100.0 (-) 100.0 100.00 100.0	n=1 20.0 (-) 20.0 20.00 20.0	NE

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.41. Pediatric Hemophilia Activities List (pedHAL) - Self care-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=12 77.2 (23.00) 67.8 81.11 94.3	-	-
OP Day 180	n=12 78.0 (20.00) 68.9 81.11 93.3	n=12 0.8 (31.73) -15.6 -2.22 14.4	-1.7 (-20.0, 24.4)
ATP Baseline	n=12 68.3 (31.23) 43.3 76.67 96.7	-	-
ATP Day 180	n=11 81.4 (31.29) 77.8 97.78 100.0	n=10 12.9 (22.75) 0.0 5.56 35.6	18.3 (-6.7, 37.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	10.0 (-32.2, 48.8)
ATP Day 360	n=11 86.3 (26.64) 91.1 100.00 100.0	n=11 18.4 (20.25) 0.0 17.78 26.7	0.4180 19.4 (10.0, 38.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.41. Pediatric Hemophilia Activities List (pedHAL) - Self care-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 76.0 (24.96) 60.0 81.11 97.5	-	-
OP Day 180	n=10 77.3 (22.04) 62.2 82.22 97.8	n=10 1.4 (34.79) -20.0 -2.22 22.2	-1.1 (-34.6, 32.4)
ATP Baseline	n=10 65.8 (33.88) 42.2 68.89 100.0	-	-
ATP Day 180	n=9 78.3 (34.03) 77.8 97.78 100.0	n=9 13.3 (24.09) 0.0 4.44 35.6	21.1 (-8.6, 50.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	20.1 (-31.1, 71.3)
ATP Day 360	n=9 83.2 (28.81) 91.1 97.78 100.0	n=9 18.3 (22.63) 0.0 4.44 26.7	0.4027 26.5 (-0.1, 53.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.41. Pediatric Hemophilia Activities List (pedHAL) - Self care- (Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=2 83.3 (11.00) 75.6 83.33 91.1	-	-
OP Day 180	n=2 81.1 (1.57) 80.0 81.11 82.2	n=2 -2.2 (12.57) -11.1 -2.22 6.7	-6.5 (-16.6, 3.6)
ATP Baseline	n=2 81.1 (1.57) 80.0 81.11 82.2	-	-
ATP Day 180	n=2 95.6 (6.29) 91.1 95.56 100.0	n=1 8.9 (-) 8.9 8.89 8.9	17.2 (10.9, 23.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	23.7 (7.2, 40.1) 0.0093
ATP Day 360	n=2 100.0 (0.00) 100.0 100.00 100.0	n=2 18.9 (1.57) 17.8 18.89 20.0	NE

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.42. Pediatric Hemophilia Activities List (pedHAL) - Household tasks-(Normalized).
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=10 67.3 (33.33) 40.0 66.67 100.0	-	-
OP Day 180	n=12 68.9 (26.72) 46.7 73.33 93.3	n=10 3.3 (36.28) -20.0 0.00 20.0	1.7 (-40.0, 43.3)
ATP Baseline	n=12 59.4 (33.08) 43.3 66.67 80.0	-	-
ATP Day 180	n=9 68.9 (33.00) 53.3 80.00 100.0	n=8 15.8 (17.80) 0.0 16.67 20.0	18.3 (13.3, 53.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	23.3 (-43.3, 50.0)
ATP Day 360	n=10 81.3 (29.11) 73.3 100.00 100.0	n=10 16.7 (15.15) 0.0 20.00 26.7	0.3594 25.0 (6.7, 46.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.42. Pediatric Hemophilia Activities List (pedHAL) - Household tasks-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=8 70.8 (35.27) 50.0 80.00 100.0	-	-
OP Day 180	n=10 68.7 (29.15) 40.0 73.33 100.0	n=8 0.0 (40.00) -20.0 0.00 10.0	3.4 (-48.8, 55.6)
ATP Baseline	n=10 63.3 (29.69) 46.7 66.67 80.0	-	-
ATP Day 180	n=7 66.7 (35.28) 46.7 80.00 100.0	n=7 10.5 (10.08) 0.0 13.33 20.0	17.5 (8.0, 27.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	14.7 (-35.2, 64.6)
ATP Day 360	n=9 79.3 (30.08) 73.3 100.00 100.0	n=9 16.3 (16.02) 0.0 20.00 26.7	0.5270 19.6 (4.2, 35.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 Inhibitor with On-Demand at OP
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 TABLE 6.1.42. Pediatric Hemophilia Activities List (pedHAL) - Household tasks-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=2 53.3 (28.28) 33.3 53.33 73.3	-	-
OP Day 180	n=2 70.0 (14.14) 60.0 70.00 80.0	n=2 16.7 (14.14) 6.7 16.67 26.7	11.9 (0.5, 23.3)
ATP Baseline	n=2 40.0 (56.57) 0.0 40.00 80.0	-	-
ATP Day 180	n=2 76.7 (33.00) 53.3 76.67 100.0	n=1 53.3 (-) 53.3 53.33 53.3	29.3 (10.4, 48.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	17.4 (9.8, 25.0) 0.0005
ATP Day 360	n=1 100.0 (-) 100.0 100.00 100.0	n=1 20.0 (-) 20.0 20.00 20.0	NE

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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Datacut: B7841005 (29APR2025)
TABLE 6.1.43. Pediatric Hemophilia Activities List (pedHAL) - Leisure activities and sports-(Normalized).
Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=11			
OP Baseline	n=9 41.6 (29.53) 21.8 34.55 54.5	-	-
OP Day 180	n=11 39.8 (25.71) 21.8 40.00 50.9	n=9 -0.8 (21.74) -12.7 0.00 14.5	0.7 (-20.7, 20.9)
ATP Baseline	n=11 48.6 (25.73) 30.9 56.36 60.0	-	-
ATP Day 180	n=8 45.8 (31.16) 15.5 45.55 75.7	n=8 -6.7 (21.91) -19.1 -6.36 7.4	-0.9 (-20.9, 13.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	0.9 (-28.7, 12.3)
ATP Day 360	n=8 52.6 (36.72) 13.4 53.30 87.5	n=8 3.3 (27.19) -10.9 3.36 21.7	0.9102 1.2 (-21.8, 22.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.
Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).
Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.43. Pediatric Hemophilia Activities List (pedHAL) - Leisure activities and sports-(Normalized)
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=9			
OP Baseline	n=8 38.3 (29.73) 17.3 30.91 51.3	-	-
OP Day 180	n=9 43.6 (27.15) 29.1 48.89 50.9	n=8 4.7 (15.31) -7.3 1.45 18.2	6.4 (-9.2, 22.0)
ATP Baseline	n=9 49.3 (27.77) 40.0 56.36 58.2	-	-
ATP Day 180	n=7 51.3 (29.16) 16.4 49.09 82.2	n=7 -4.3 (22.49) -14.5 1.82 12.7	-2.3 (-22.1, 17.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-5.6 (-29.6, 18.4)
ATP Day 360	n=8 52.6 (36.72) 13.4 53.30 87.5	n=8 3.3 (27.19) -10.9 3.36 21.7	0.6157 4.5 (-19.8, 28.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.43. Pediatric Hemophilia Activities List (pedHAL) - Leisure activities and sports-(Normalized)
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=2			
OP Baseline	n=1 68.0 (-) 68.0 68.00 68.0	-	-
OP Day 180	n=2 22.7 (1.29) 21.8 22.73 23.6	n=1 -44.4 (-) -44.4 -44.36 -44.4	NE
ATP Baseline	n=2 45.5 (20.57) 30.9 45.45 60.0	-	-
ATP Day 180	n=1 7.3 (-) 7.3 7.27 7.3	n=1 -23.6 (-) -23.6 -23.64 -23.6	NE
Treatment difference ATP Day 180 vs. OP Day 180	-	-	NE
ATP Day 360	n=0		NE

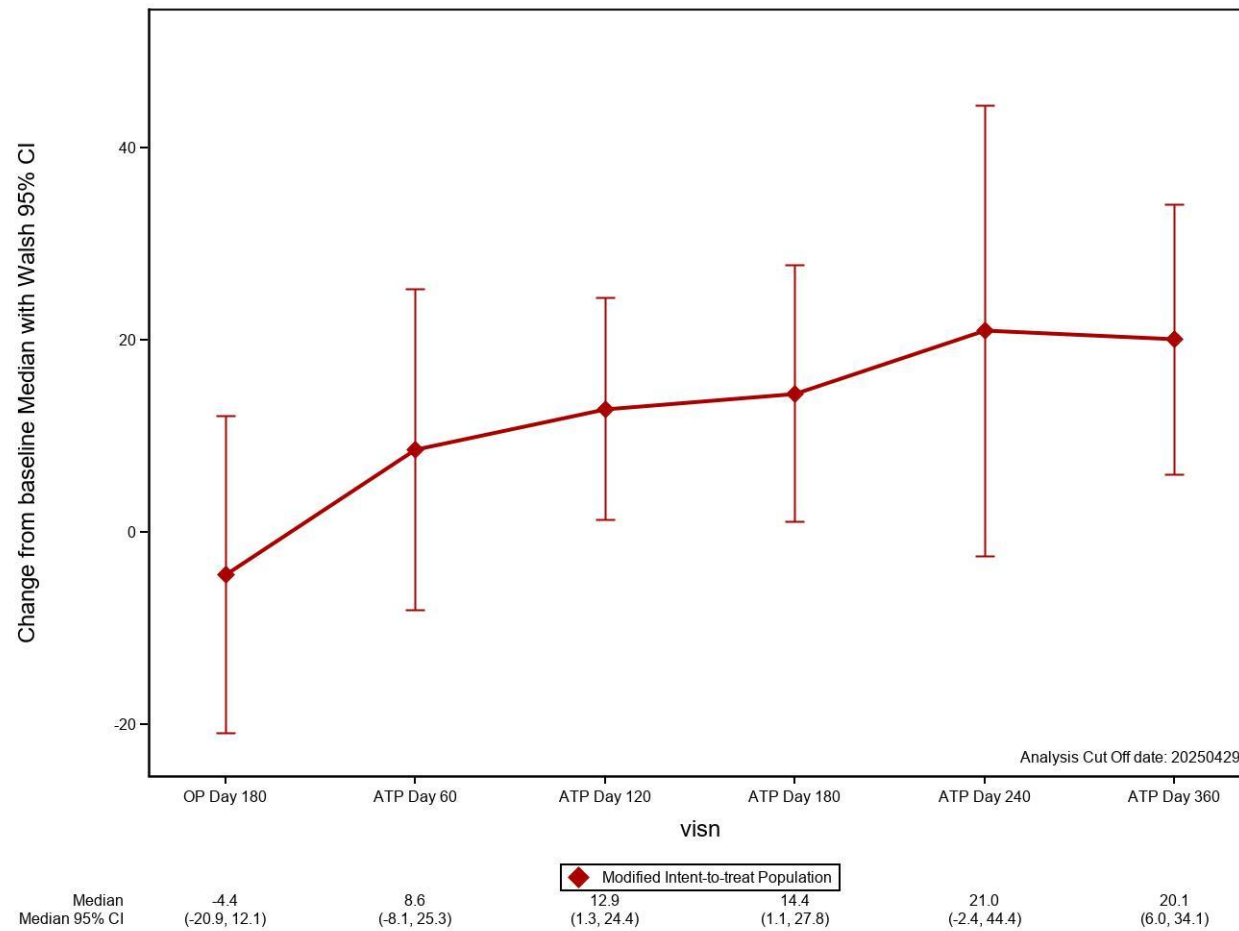
ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

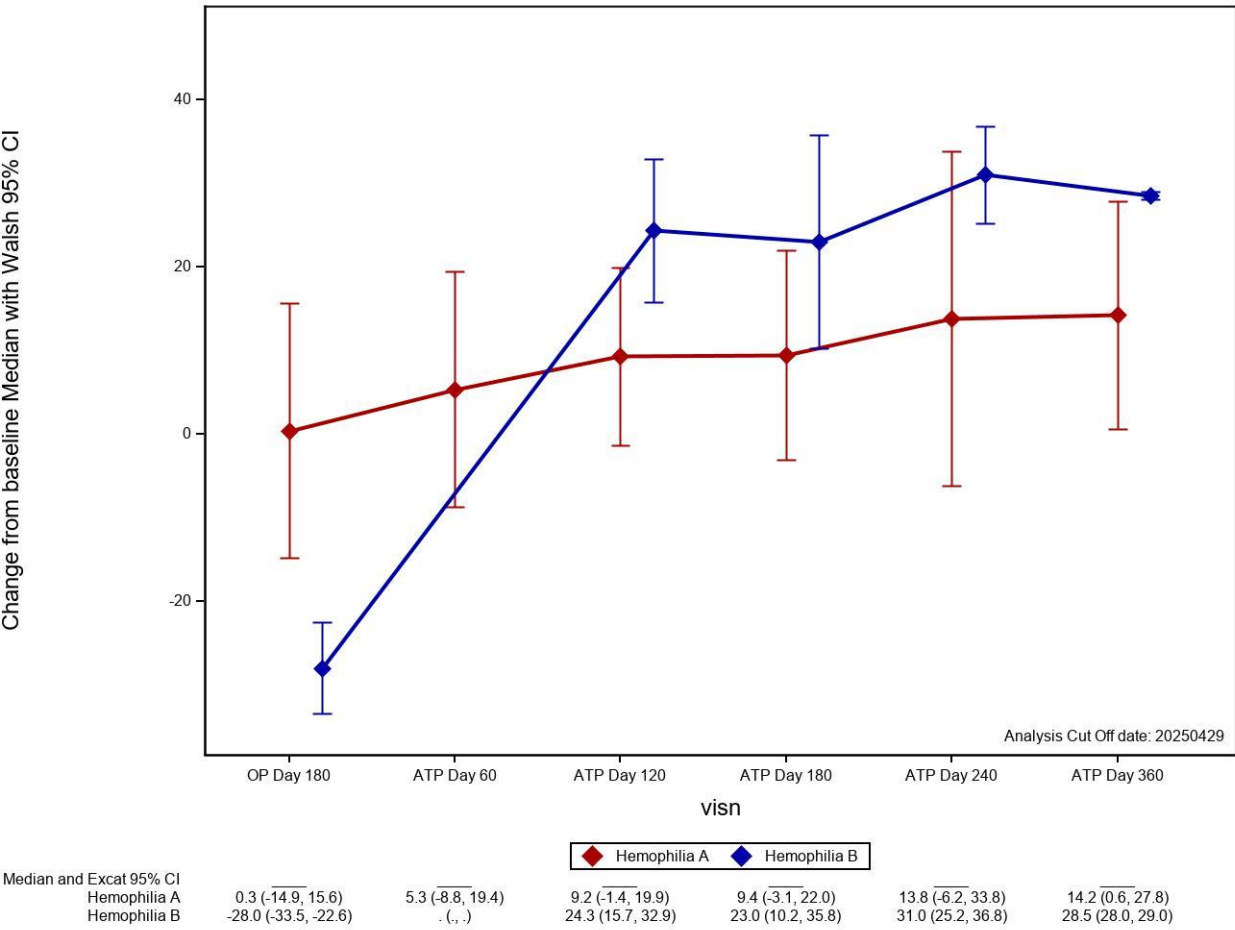
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.36.1: Pediatric Hemophilia Activities List (pedHAL) - Total Score-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



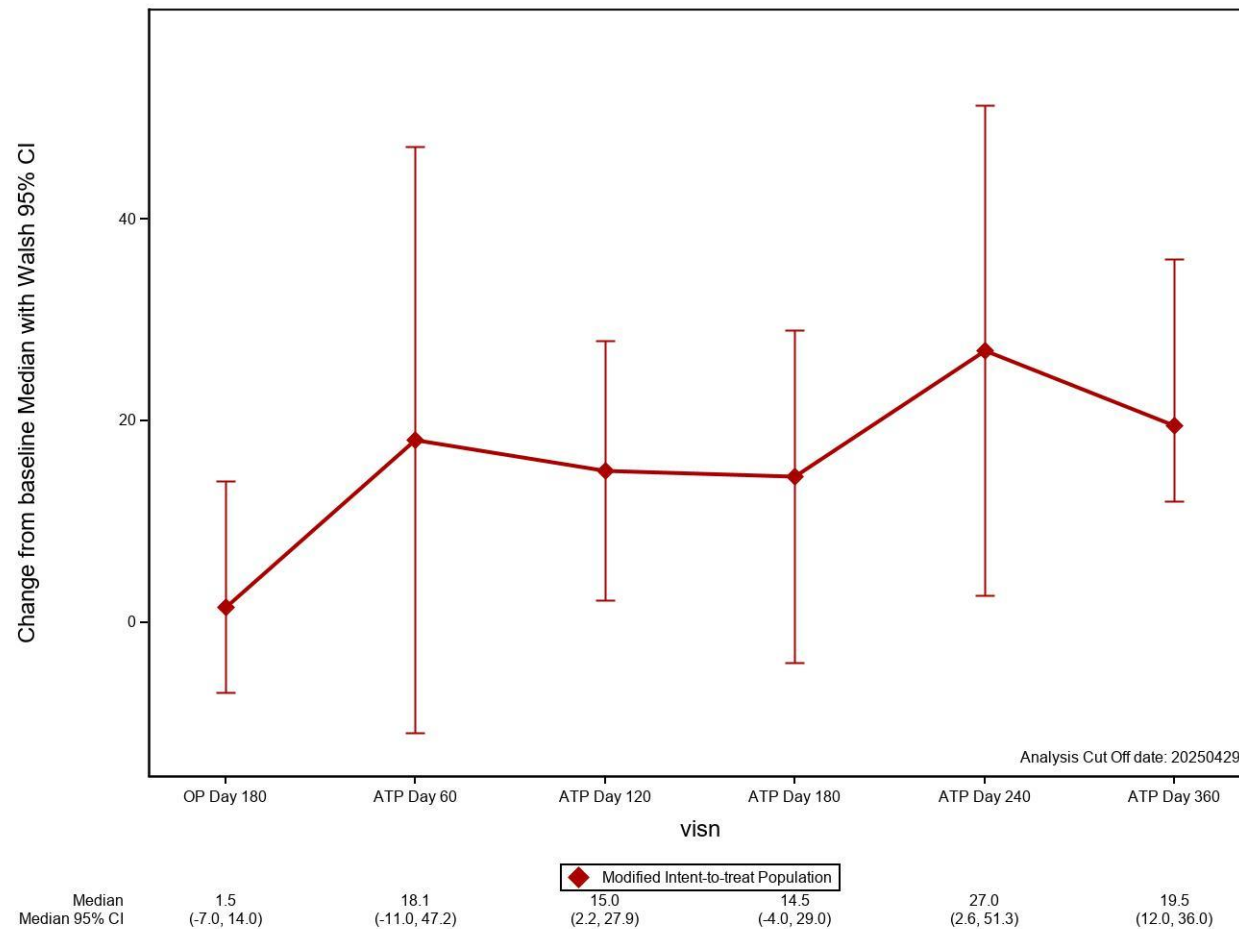
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.36.2: Pediatric Hemophilia Activities List (pedHAL) - Total Score-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



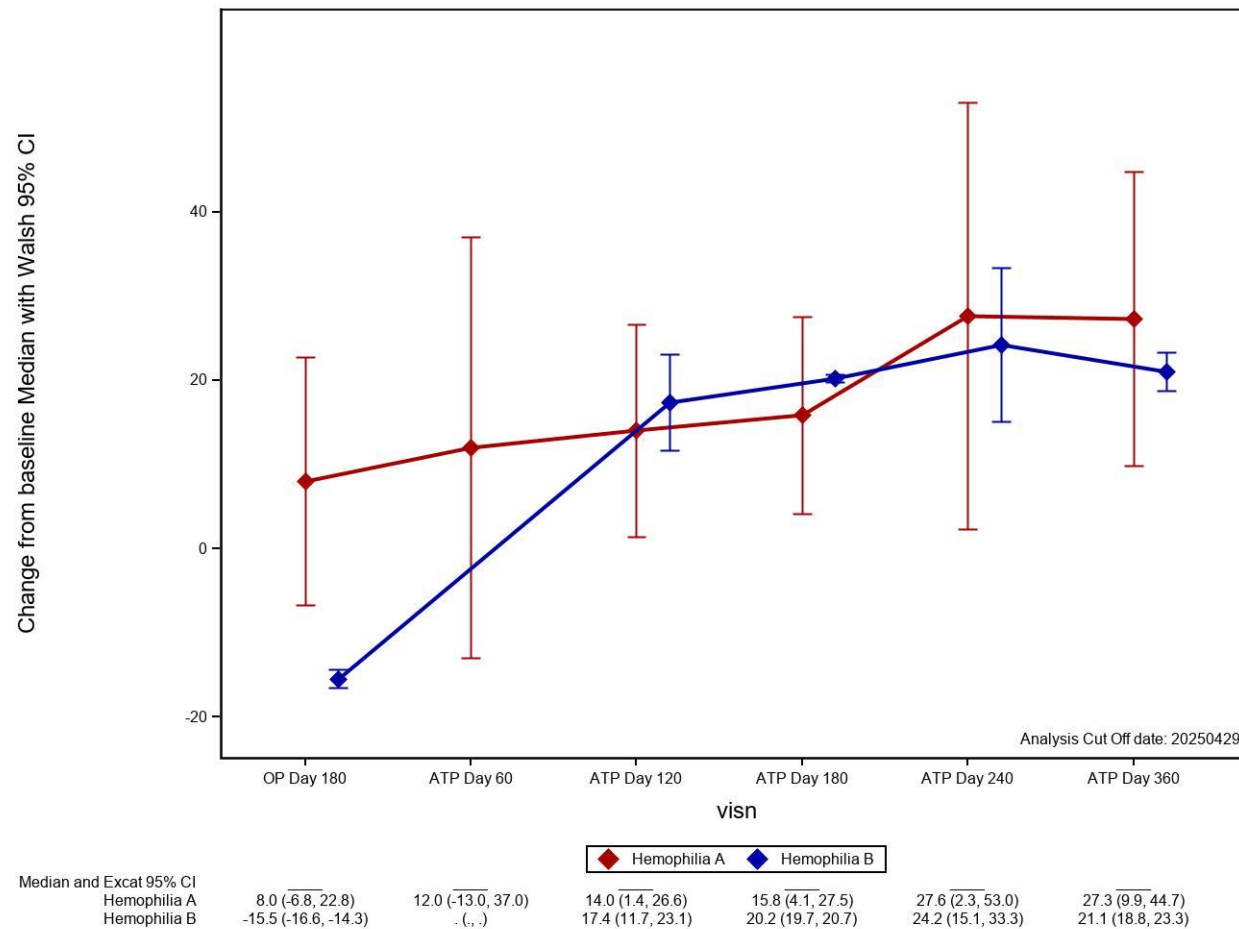
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.37.1: Pediatric Hemophilia Activities List (pedHAL) - Lying / sitting / kneeling / standing-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



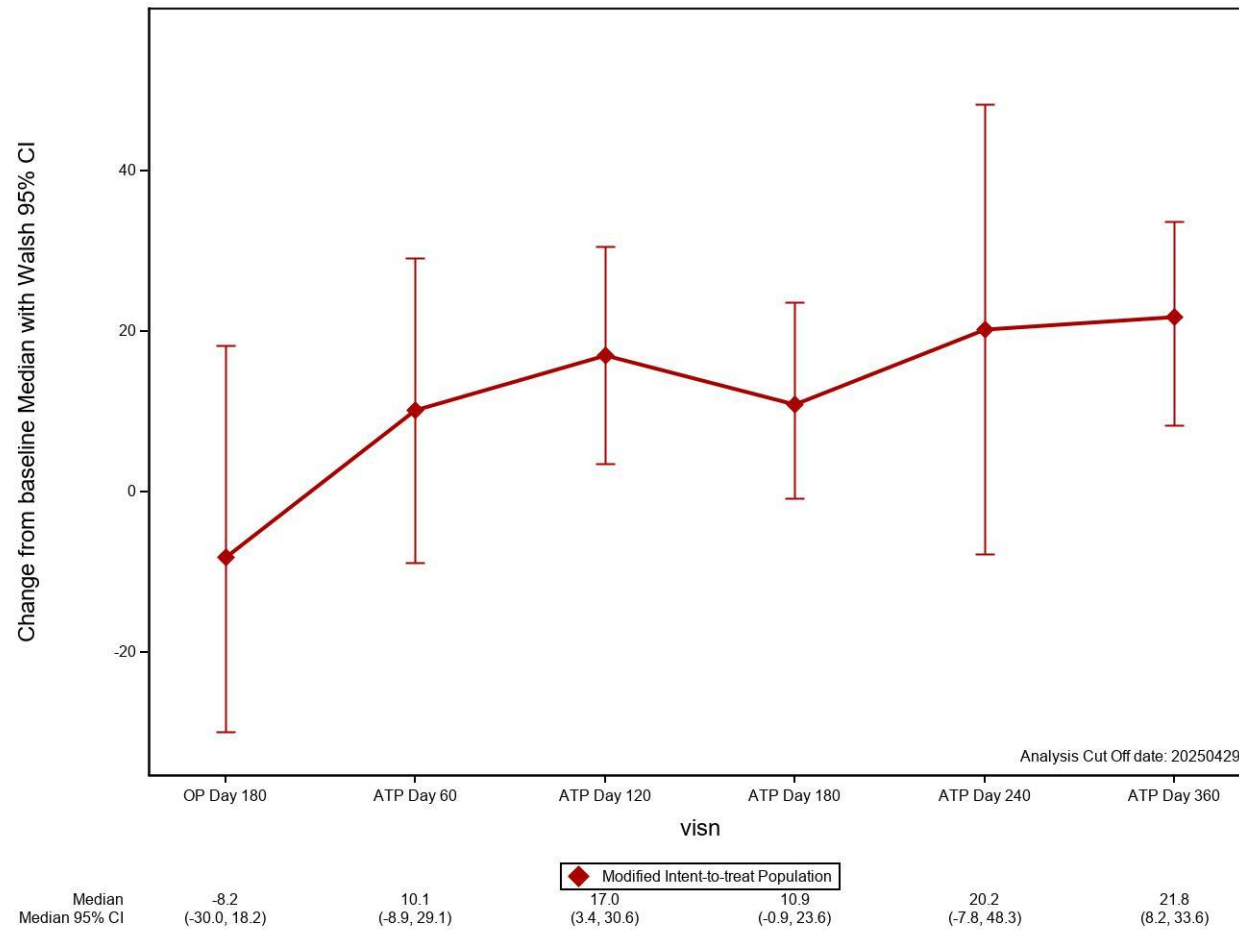
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.37.2: Pediatric Hemophilia Activities List (pedHAL) - Lying / sitting / kneeling / standing-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



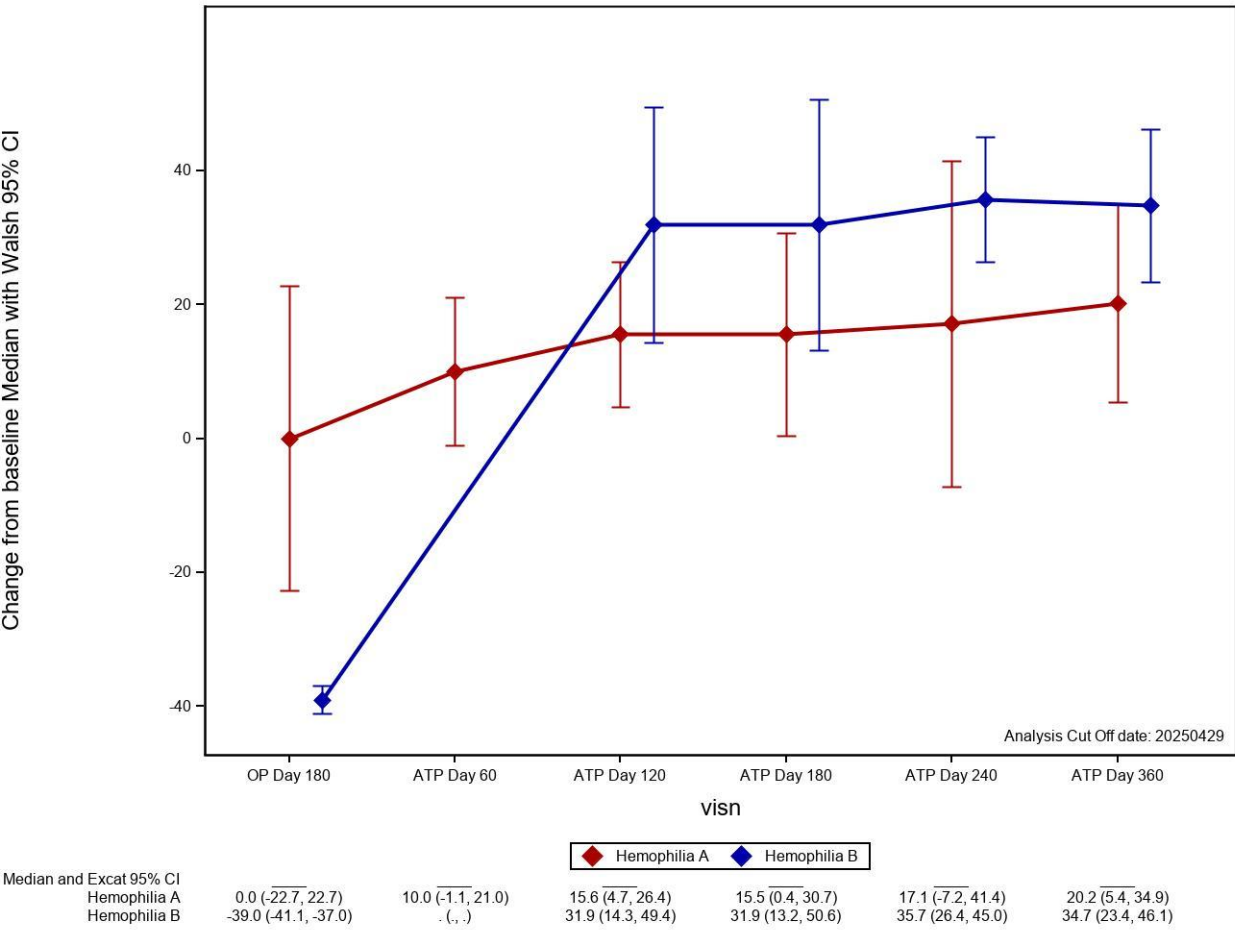
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.38.1: Pediatric Hemophilia Activities List (pedHAL) - Functions of the legs-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



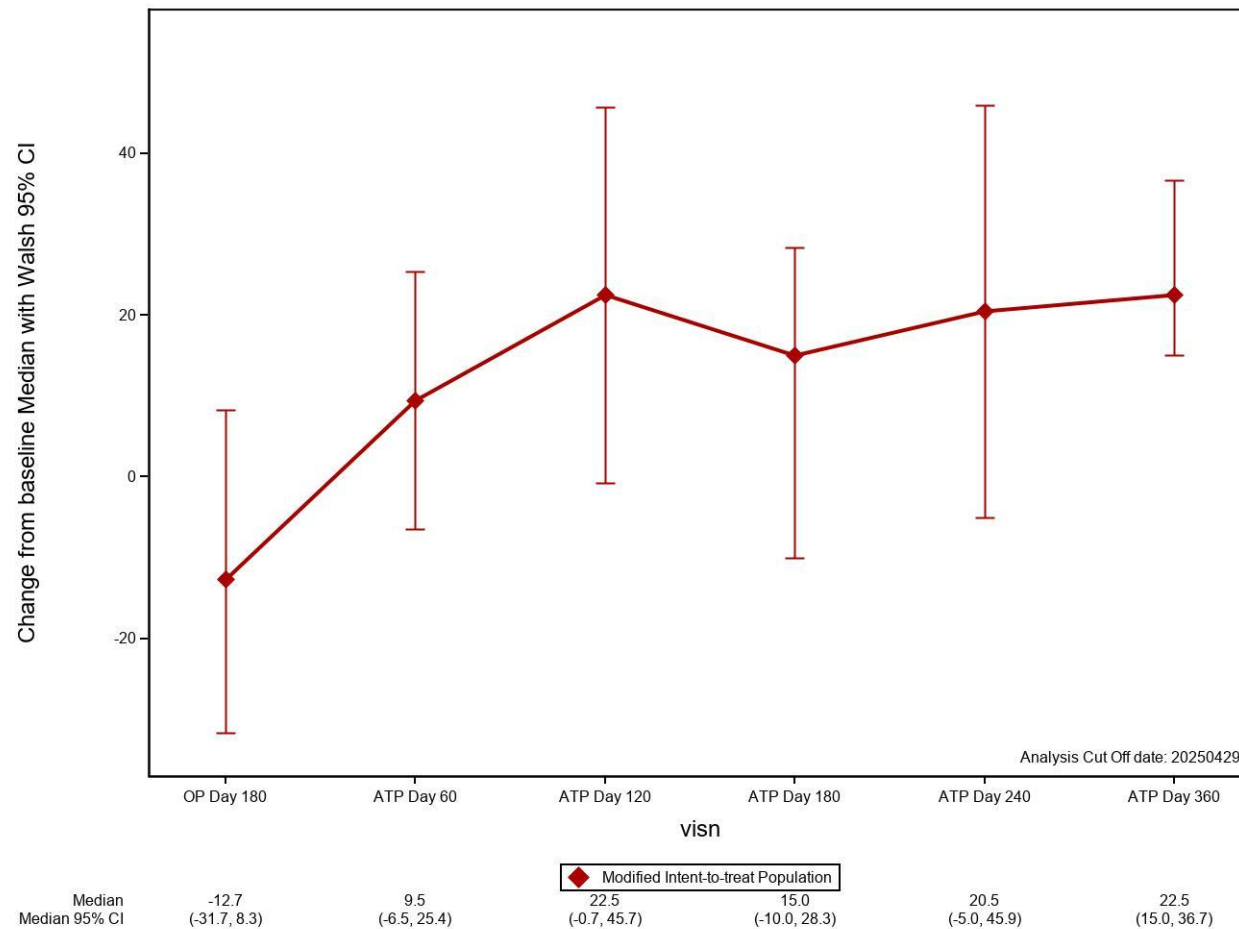
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.38.2: Pediatric Hemophilia Activities List (pedHAL) - Functions of the legs-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



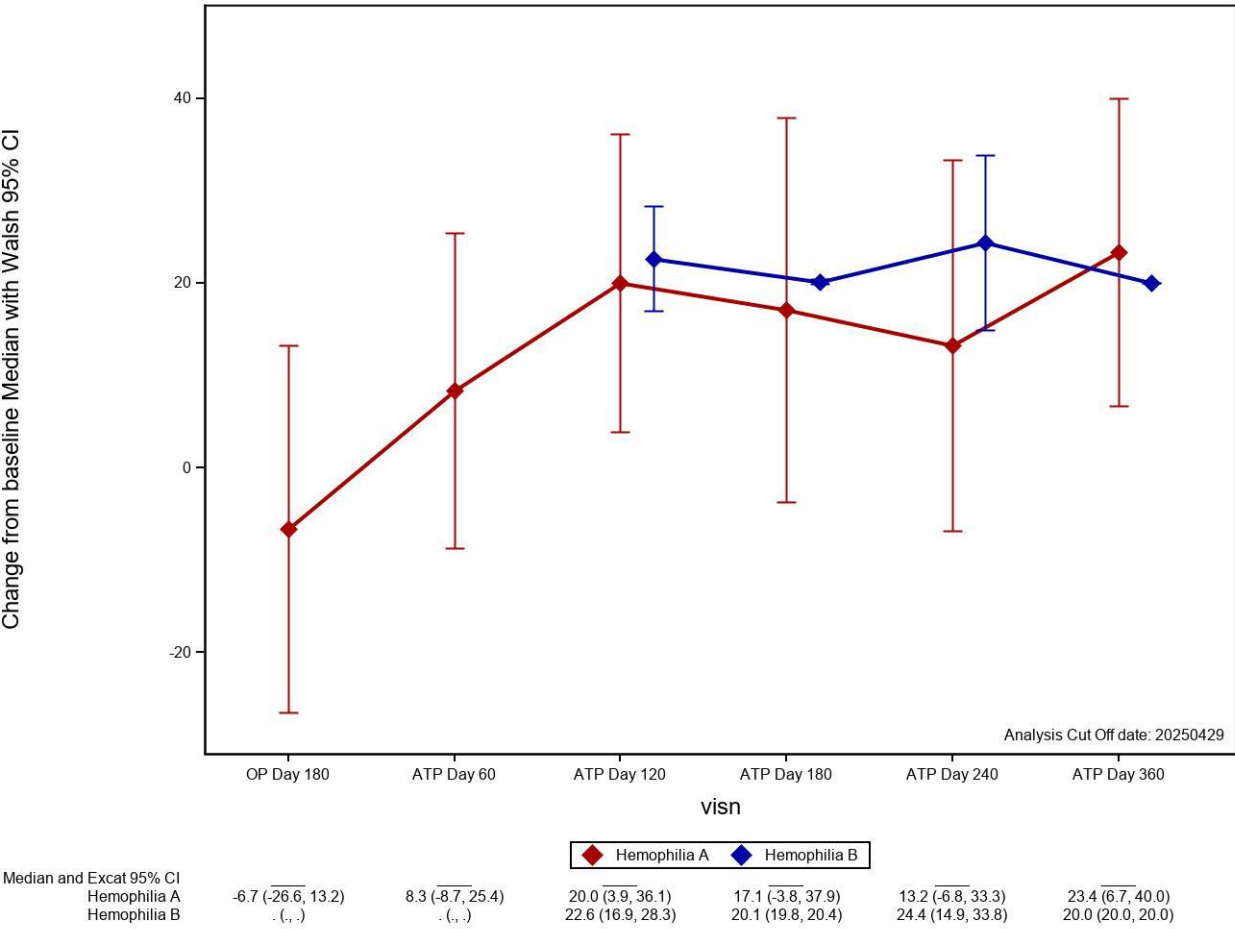
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.39.1: Pediatric Hemophilia Activities List (pedHAL) - Functions of the arms- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



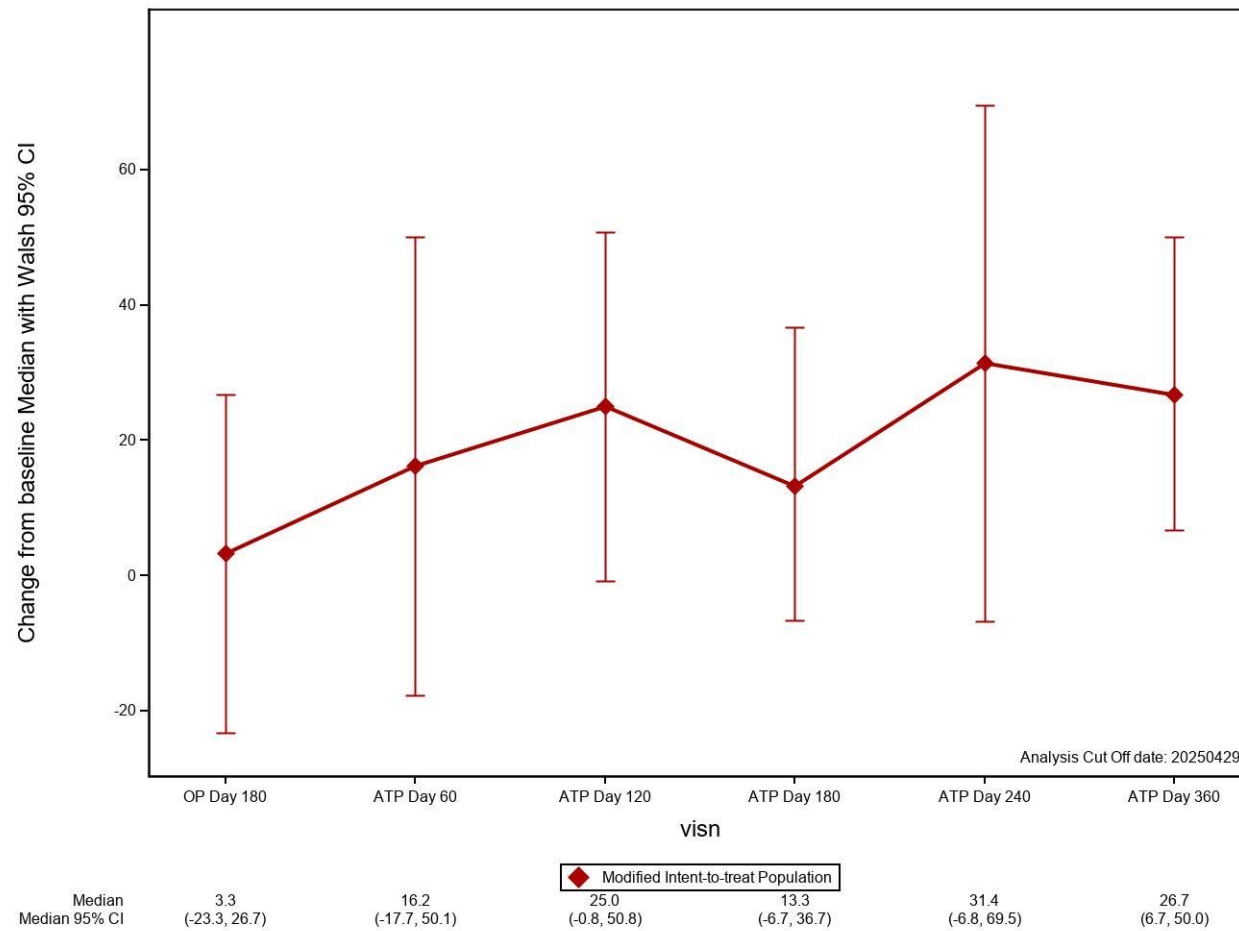
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.39.2: Pediatric Hemophilia Activities List (pedHAL) - Functions of the arms-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



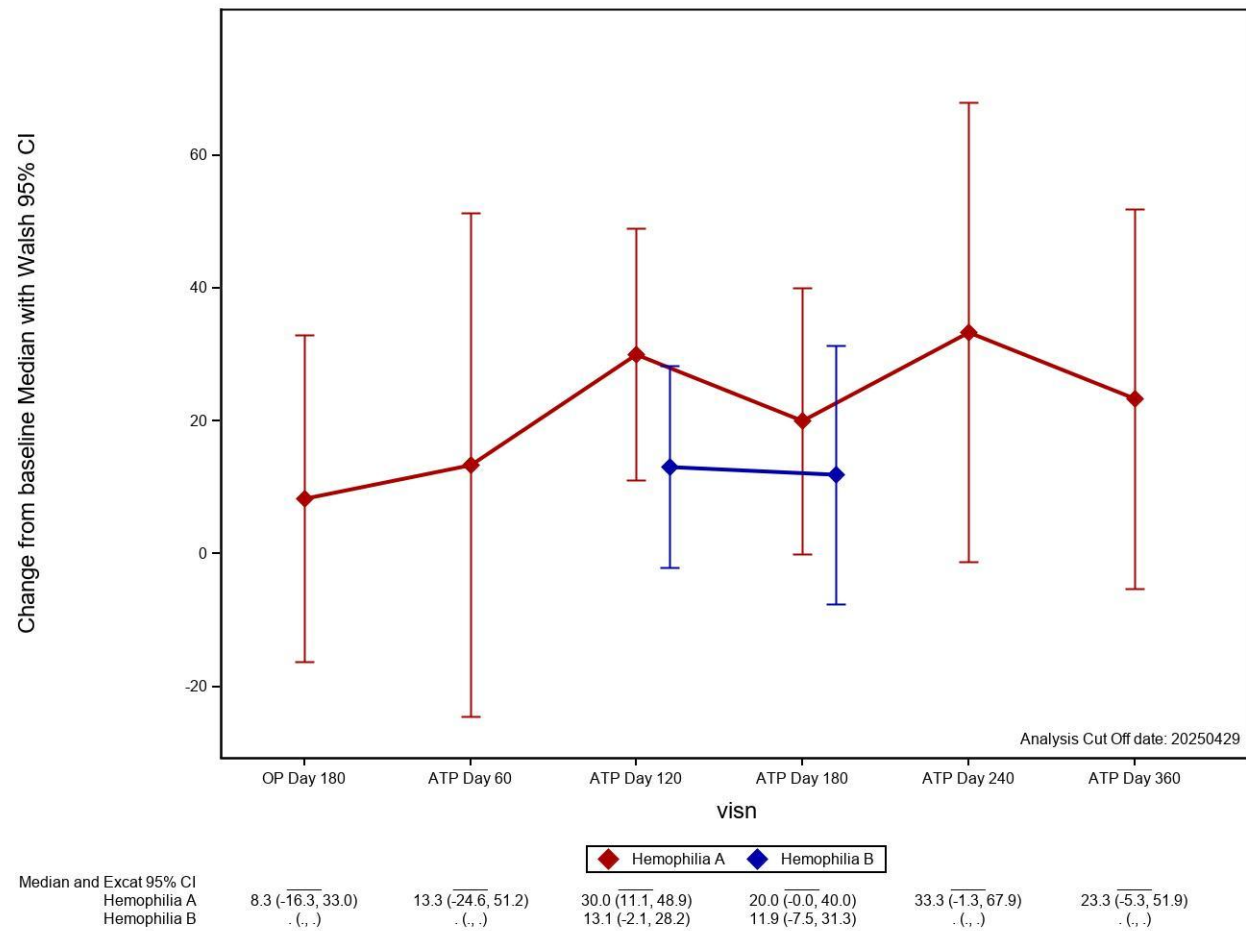
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.40.1: Pediatric Hemophilia Activities List (pedHAL) - Use of transportation-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



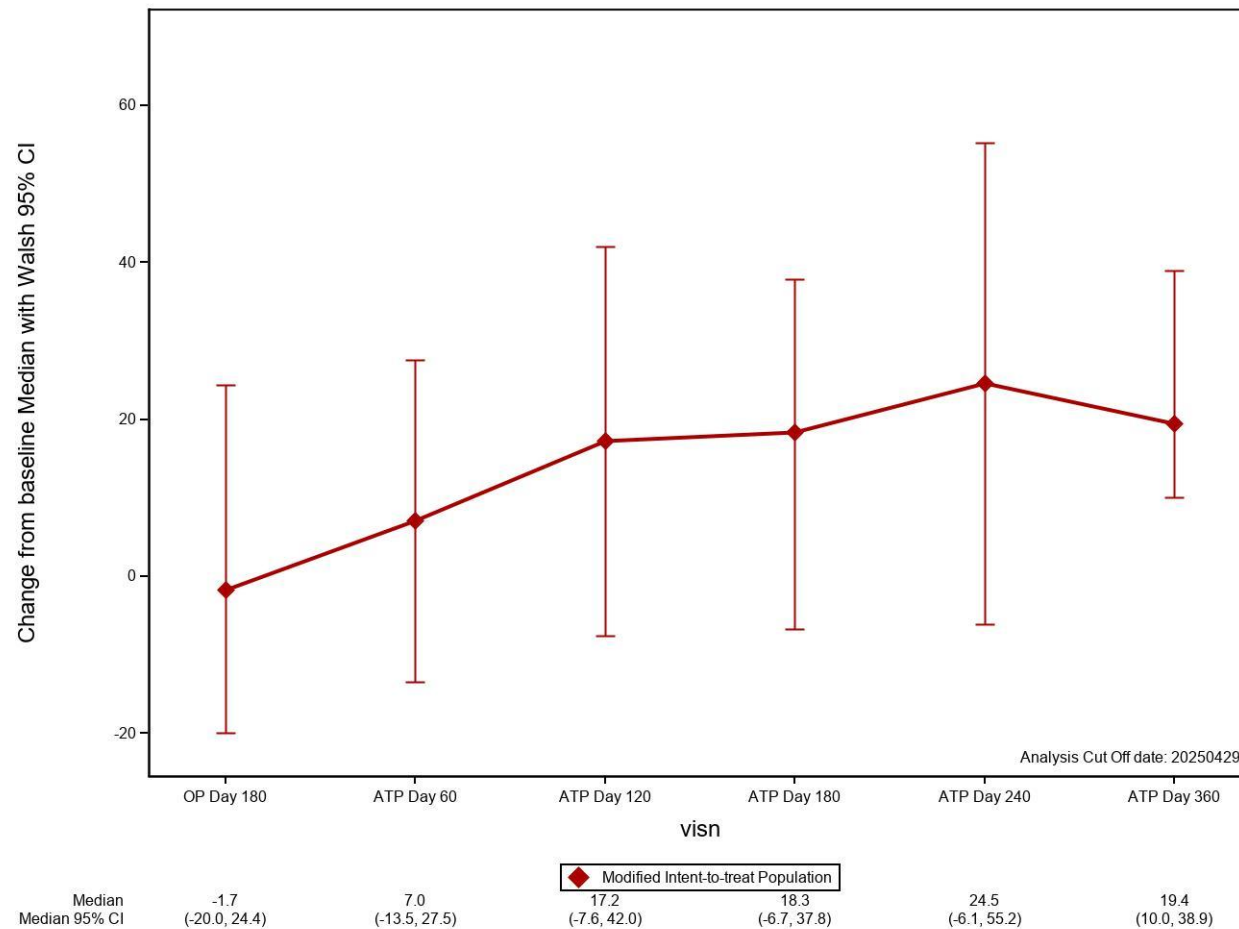
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.40.2: Pediatric Hemophilia Activities List (pedHAL) - Use of transportation-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



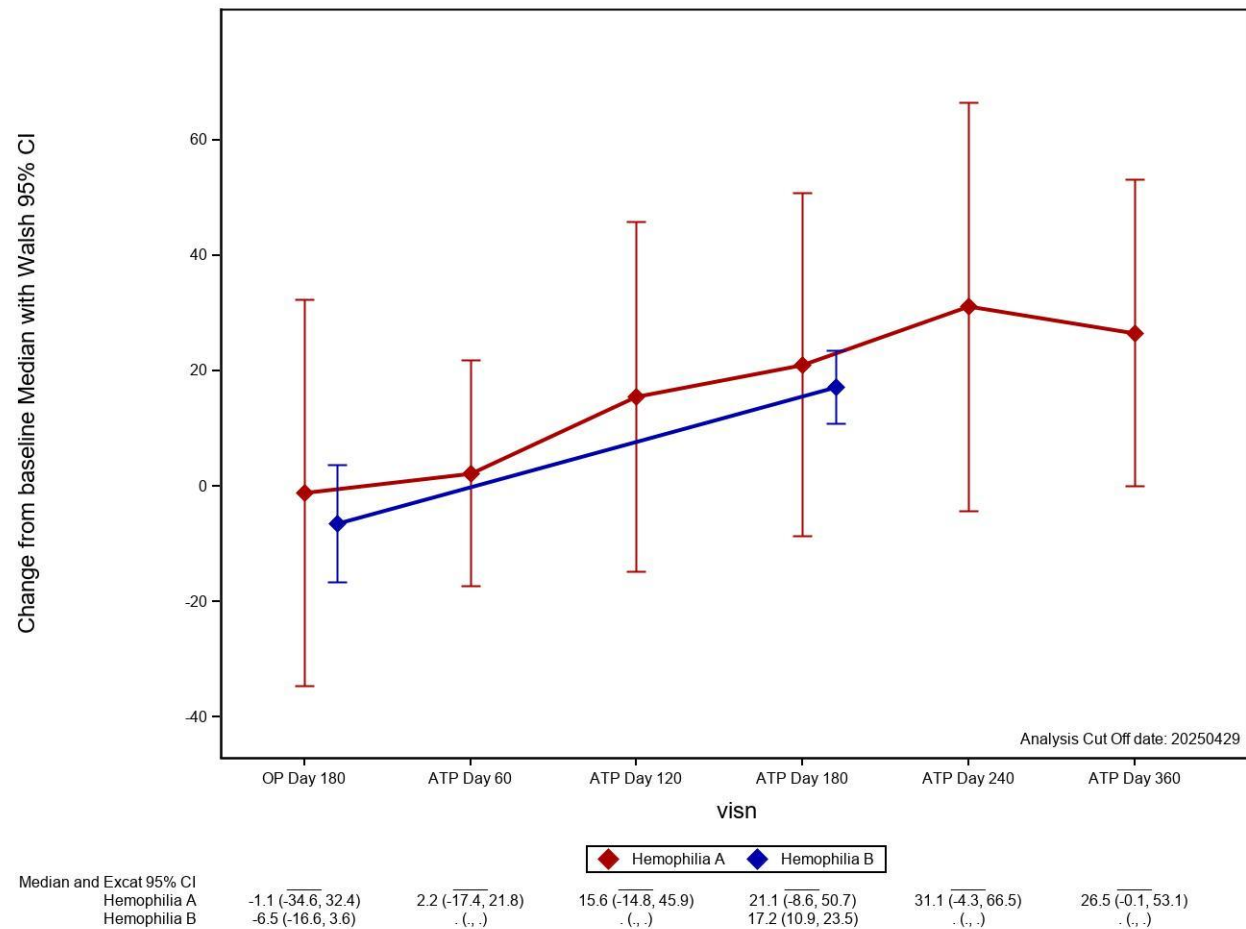
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.41.1: Pediatric Hemophilia Activities List (pedHAL) - Self care-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



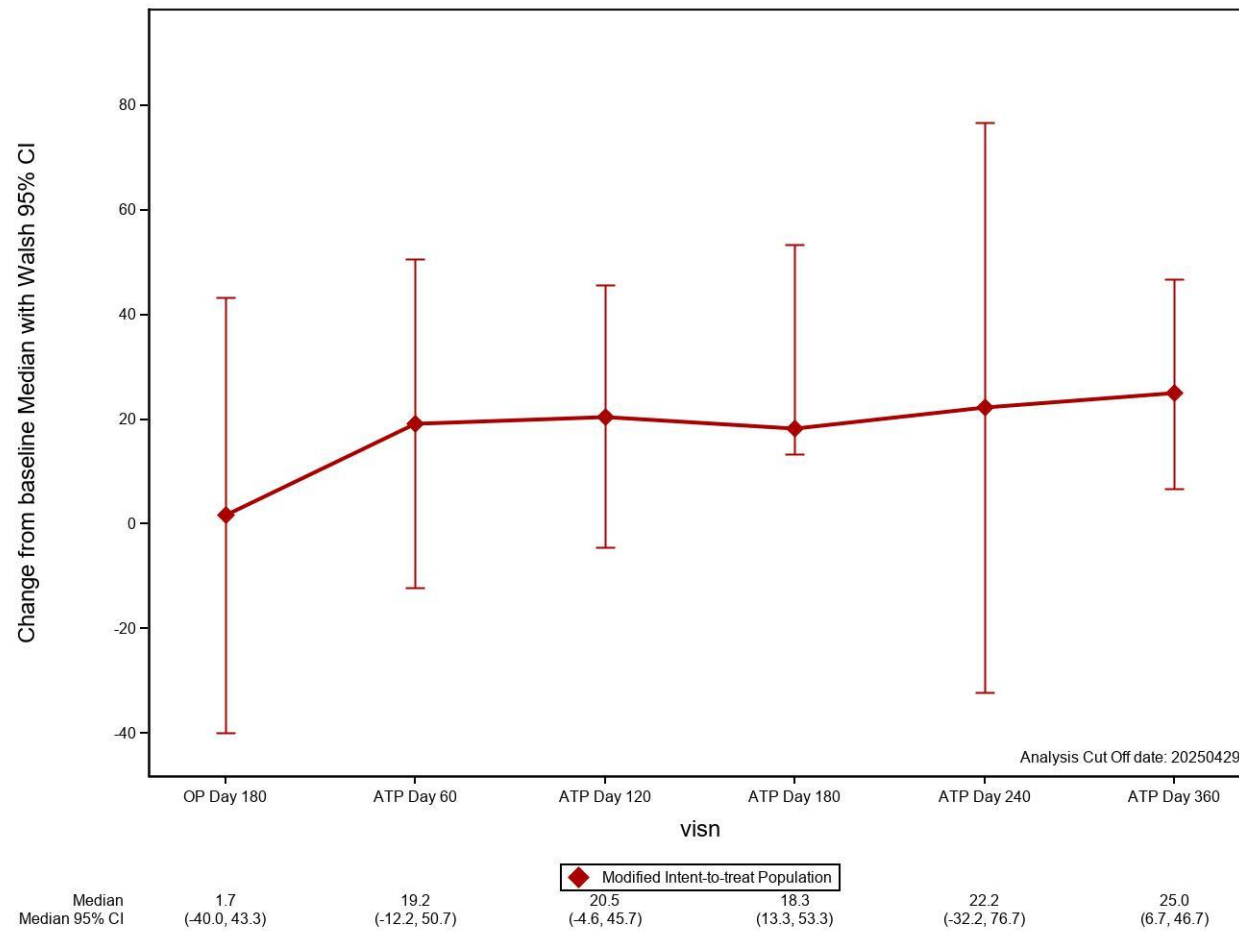
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.41.2: Pediatric Hemophilia Activities List (pedHAL) - Self care- (Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



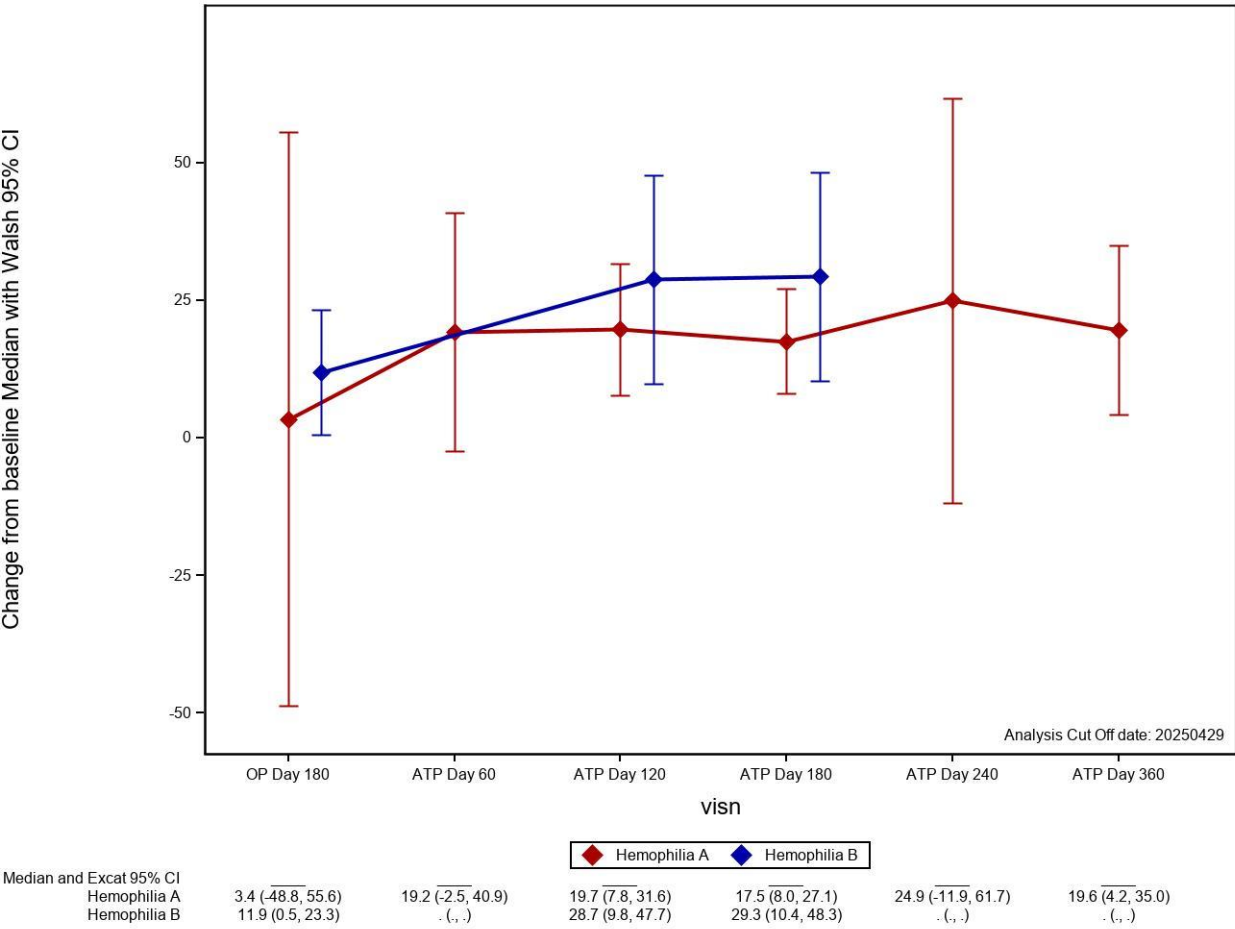
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.42.1: Pediatric Hemophilia Activities List (pedHAL) - Household tasks-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



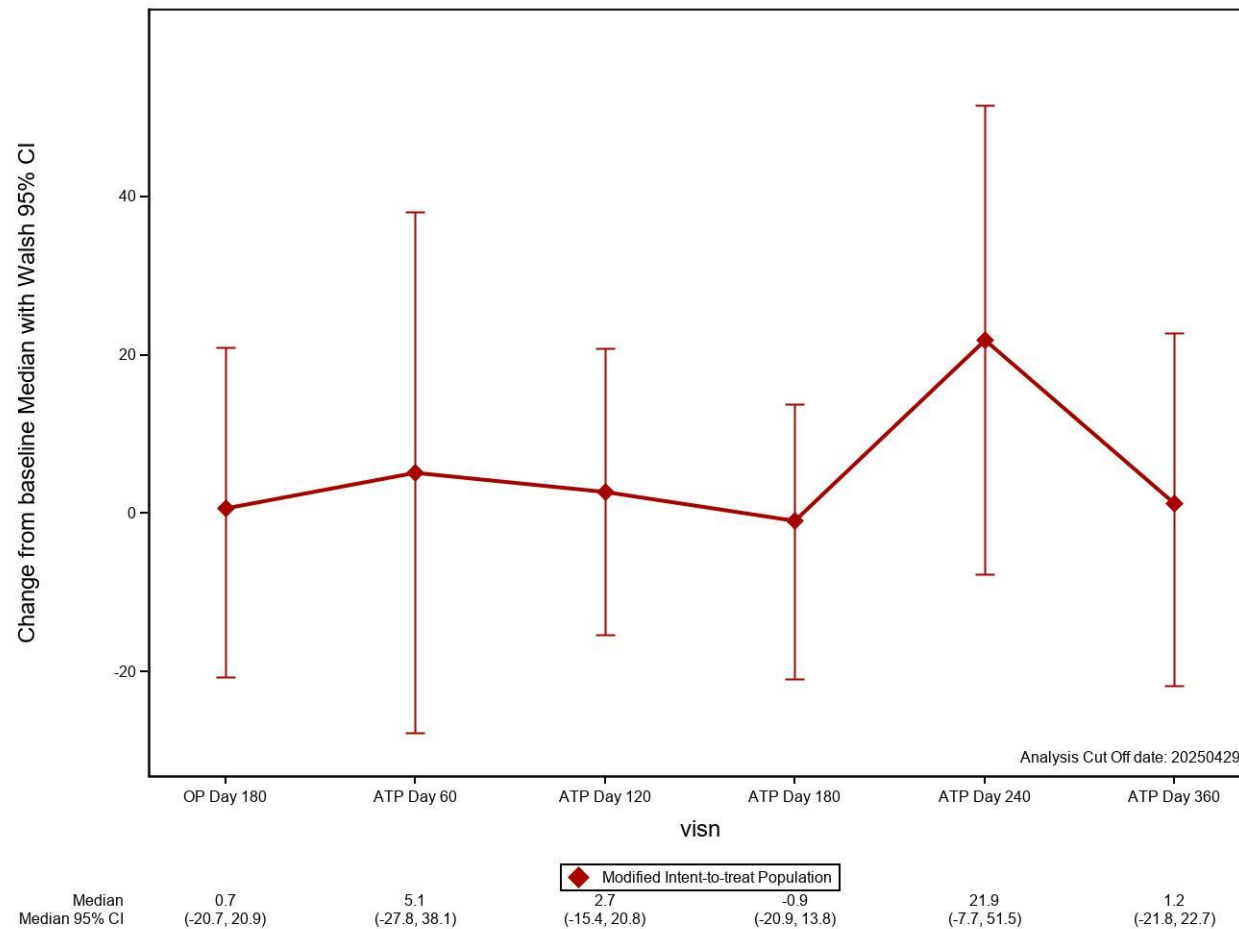
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.42.2: Pediatric Hemophilia Activities List (pedHAL) - Household tasks-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



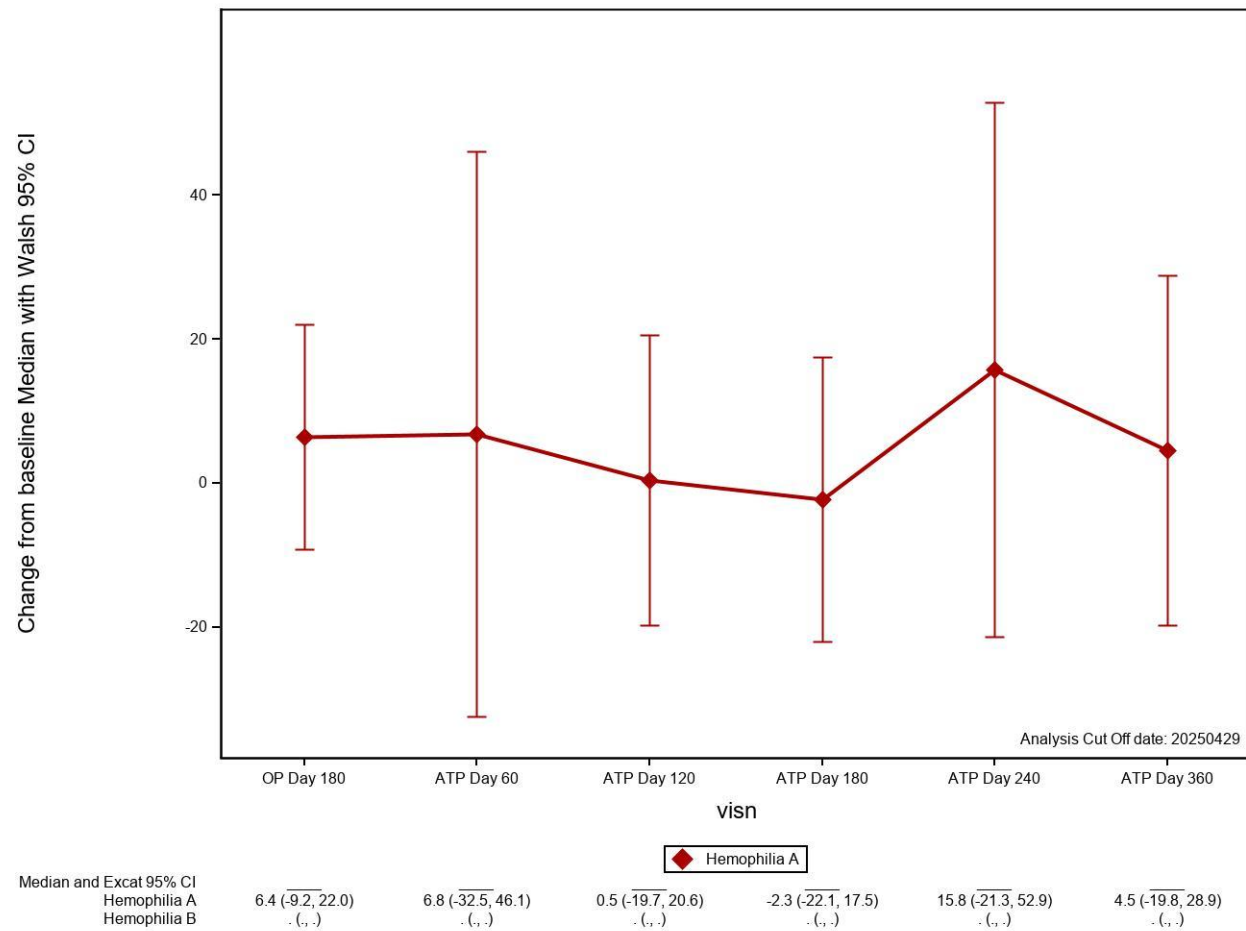
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.43.1: Pediatric Hemophilia Activities List (pedHAL) - Leisure activities and sports-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



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

FIGURE .6.2.43.2: Pediatric Hemophilia Activities List (pedHAL) - Leisure activities and sports-(Normalized) - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



6.3 HJHS

6.3.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.1.9. Hemophilia Joint Health Score (HJHS) Total Score response (≥ 15 points Improvement from baseline) analysis.

Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Overall								
-	48	2.9 (6.0%)	1.1 (2.3%)	2.0 (4.2%)	0.38 (0.03, 4.23) 0.4314	0.37 (0.03, 4.43) 0.4289	-3.8 (-12.8, 5.3) 0.4143	
Disease type								
Hemophilia A	40	1.7 (4.3%)	0.1 (0.3%)	1.0 (2.5%)	0.40 (0.03, 4.74) 0.4699	0.39 (0.03, 4.99) 0.4664	-4.0 (-13.9, 5.9) 0.4277	
Hemophilia B	8	1.2 (15.0%)	1.0 (12.5%)	1.0 (12.5%)	0.87 (0.05, 13.87) 0.9218	0.84 (0.03, 20.77) 0.9174	-2.5 (-40.3, 35.3) 0.8967	0.7062
Age group								
Adults (Ages 18 to < 75)	36	2.2 (6.1%)	1.1 (3.1%)	2.0 (5.6%)	0.49 (0.04, 5.52) 0.5670	0.48 (0.04, 5.92) 0.5657	-3.1 (-13.4, 7.3) 0.5629	
Adolescents (Ages 12 to < 18)	12	0.7 (5.8%)	0.0 (0.0%)	0.0 (0.0%)	0.62 (0.05, 8.39) 0.7156	0.58 (0.03, 10.63) 0.7105	-5.8 (-33.7, 22.0) 0.6810	0.9234
Region								
Asia	24	2.2 (9.2%)	0.0 (0.0%)	1.0 (4.2%)	-	-	-	
Europe	8	0.0 (0.0%)	0.0 (0.0%)	0.0 (0.0%)	-	-	-	
Other	16	0.7 (4.4%)	1.1 (6.9%)	1.0 (6.3%)	-	-	-	-
White Race								
White	15	0.7 (4.7%)	1.1 (7.3%)	1.0 (6.7%)	1.28 (0.08, 20.89) 0.8613	1.32 (0.06, 27.24) 0.8566	2.7 (-19.7, 25.0) 0.8144	
Other	33	2.2 (6.7%)	0.0 (0.0%)	1.0 (3.0%)	0.31 (0.03, 3.02) 0.3166	0.29 (0.03, 3.16) 0.3115	-6.7 (-18.9, 5.6) 0.2856	0.4400

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visit data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD were performed by imputation, and the results were pooled using Rubin's rules.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

6.3.2 Sensitivitätsanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 7 TABLE 6.1.46. Hemophilia Joint Health Score (HJHS) total score.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=48			
OP Baseline	n=46 25.1 (19.79) 9.0 21.5 35.0	-	-
OP Day 180	n=48 23.5 (19.94) 4.3 26.4 35.0	n=46 -1.3 (13.19) -3.0 0.0 1.0	-1.1 (-4.00, 1.90)
ATP Baseline	n=46 23.4 (20.19) 4.0 25.5 35.0	-	-
ATP Day 180	n=46 22.9 (17.20) 9.0 23.5 32.0	n=44 -1.3 (10.22) -4.3 -1.0 0.0	-3.9 (-6.10, -1.70)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-2.2 (-5.70, 1.40) 0.2341
ATP Day 360	n=45 22.1 (18.08) 4.0 21.0 37.0	n=43 -1.7 (14.75) -7.0 -2.0 0.0	-3.7 (-6.30, -1.00)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. Total Score is reported; it ranges from 0 to 124 with higher score indicating worse joint health.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.46. Hemophilia Joint Health Score (HJHS) total score
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=40			
OP Baseline	n=39 24.0 (19.09) 9.0 21.0 35.0	-	-
OP Day 180	n=40 22.2 (17.55) 4.3 25.5 35.0	n=39 -1.3 (13.24) -2.0 0.0 1.0	-0.7 (-4.13, 2.68)
ATP Baseline	n=38 22.1 (17.80) 4.0 25.5 35.0	-	-
ATP Day 180	n=39 21.1 (16.19) 7.0 21.0 32.0	n=37 -1.5 (7.59) -4.5 -1.0 0.0	-2.9 (-5.33, -0.45)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-2.1 (-5.83, 1.73)
ATP Day 360	n=38 21.0 (17.64) 4.0 17.0 36.2	n=36 -1.3 (14.56) -7.5 -3.0 0.0	0.2544 -4.3 (-7.17, -1.52)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. Total Score is reported; it ranges from 0 to 124 with higher score indicating worse joint health.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.46. Hemophilia Joint Health Score (HJHS) total score
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=8			
OP Baseline	n=7 31.3 (24.00) 7.2 33.0 52.0	-	-
OP Day 180	n=8 30.2 (29.86) 7.5 29.3 37.0	n=7 -1.2 (13.97) -7.2 -3.0 5.0	-2.3 (-15.04, 10.54)
ATP Baseline	n=8 29.4 (29.94) 7.5 26.0 37.0	-	-
ATP Day 180	n=7 33.4 (20.19) 19.6 32.0 52.0	n=7 -0.2 (20.02) -1.0 1.0 14.0	3.3 (-16.29, 22.93)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	9.2 (-16.99, 35.31)
ATP Day 360	n=7 27.7 (20.85) 0.0 36.0 44.0	n=7 -4.0 (16.70) -2.0 0.0 6.0	0.4532 3.0 (-18.00, 24.00)

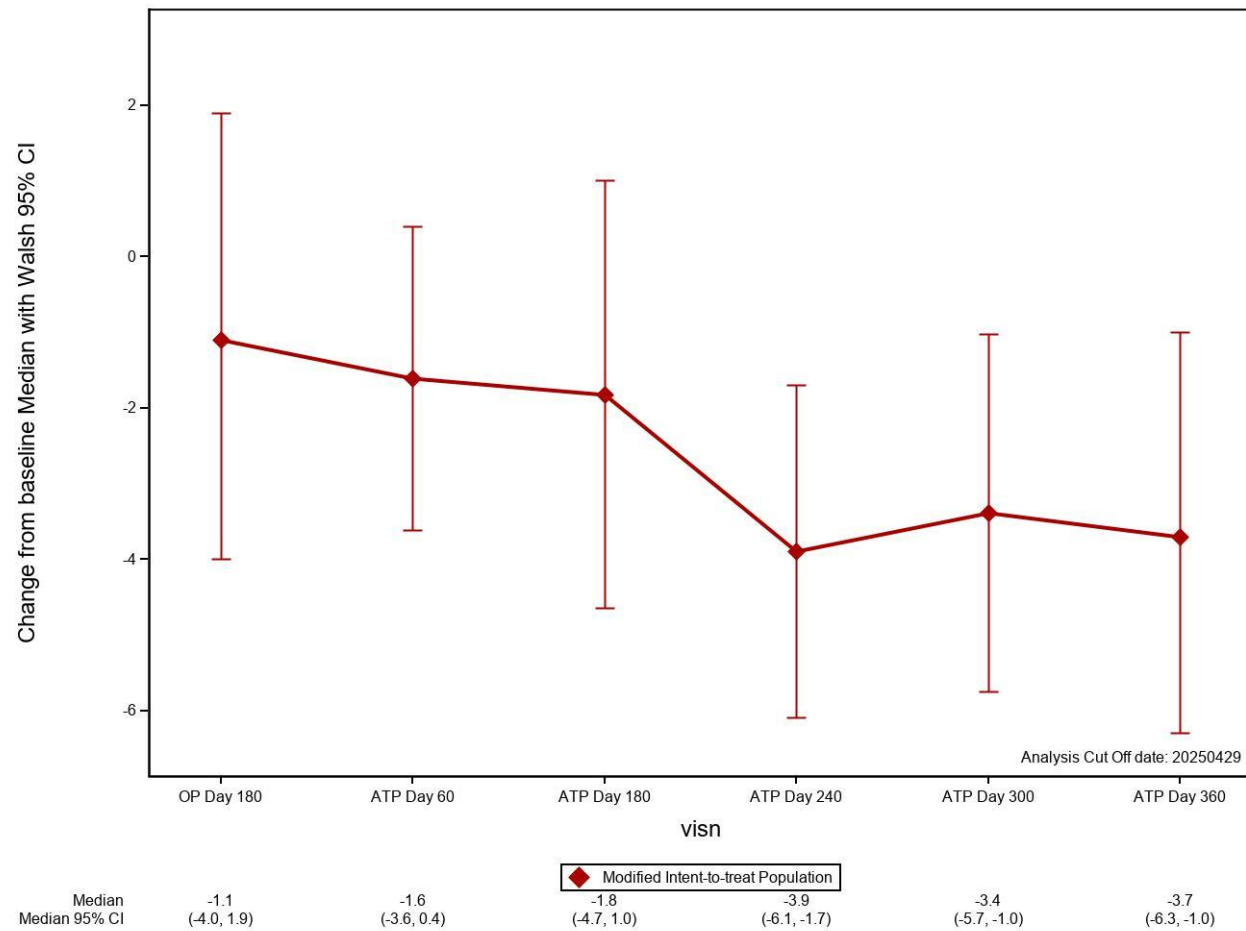
ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation. Total Score is reported; it ranges from 0 to 124 with higher score indicating worse joint health.

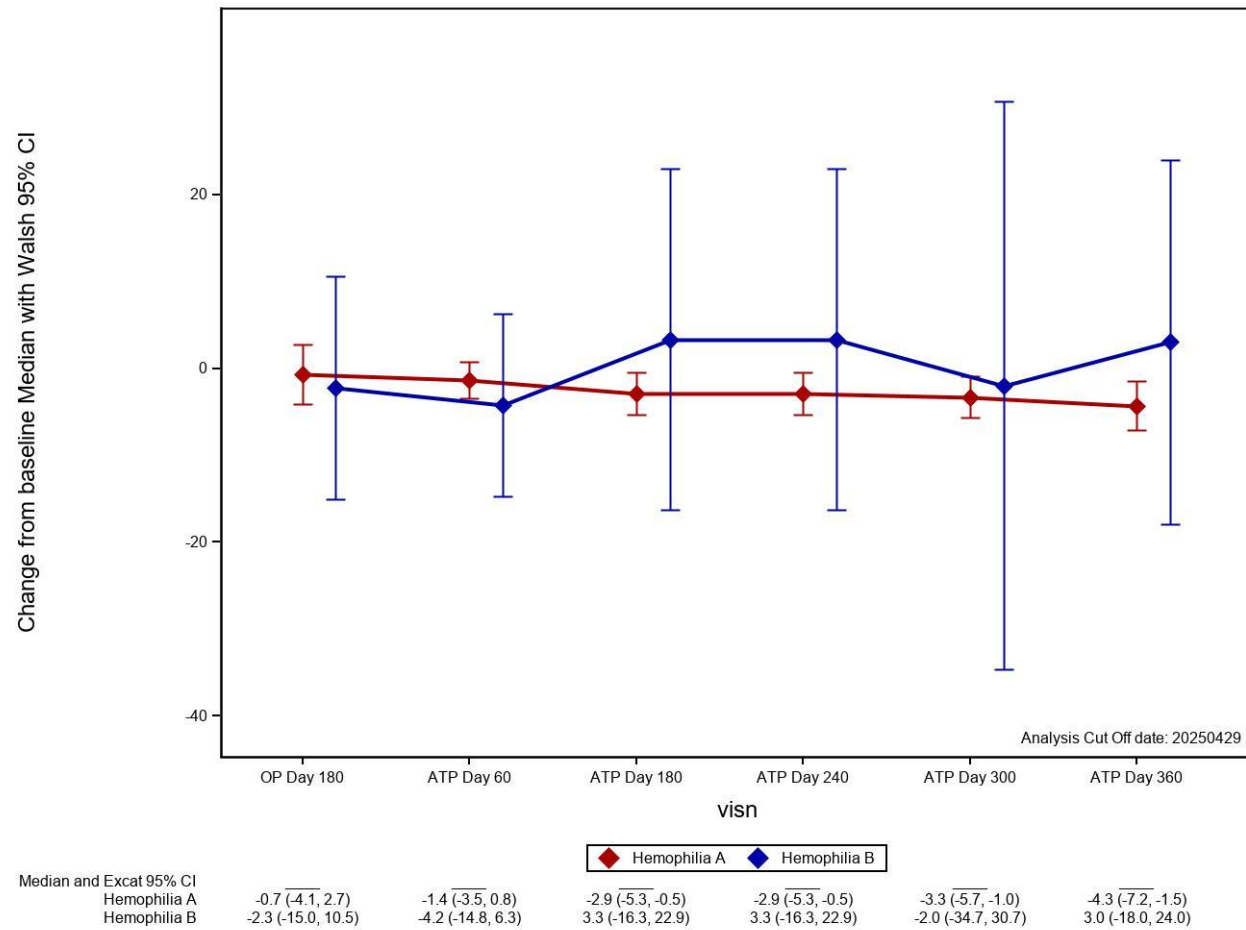
FIGURE .6.2.46.1: Hemophilia Joint Health Score (HJHS) total score - Modified Intent-to-Treat Set - Change from baseline Medians - Overall

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



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

FIGURE .6.2.46.2: Hemophilia Joint Health Score (HJHS) total score - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



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

8 Gesundheitsbezogene Lebensqualität

8.1 Haem-A-QoL

8.1.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.1.1. Haem-A-QoL Total score response (≥ 15 points Improvement from baseline) analysis.

Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Overall								
-	35	2.3 (6.6%)	13.1 (37.4%)	15.0 (42.9%)	5.80 (1.46, 23.05) 0.0126	8.66 (1.84, 40.78) 0.0064	30.9 (12.9, 48.8) 0.0007	
Disease type								
Hemophilia A	30	2.2 (7.3%)	11.1 (37.0%)	11.0 (36.7%)	5.12 (1.27, 20.68) 0.0220	7.53 (1.53, 37.00) 0.0130	29.7 (10.1, 49.3) 0.0030	
Hemophilia B	5	0.1 (2.0%)	2.0 (40.0%)	4.0 (80.0%)	2.88 (0.29, 28.52) 0.3657	5.53 (0.15, 201.48) 0.3510	38.0 (-32.9, 108.9) 0.2934	0.8788
Region								
Asia	18	0.1 (0.6%)	7.0 (38.9%)	9.0 (50.0%)	-	-	-	
Europe	5	1.0 (20.0%)	4.0 (80.0%)	3.0 (60.0%)	-	-	-	
Other	12	1.2 (10.0%)	2.1 (17.5%)	3.0 (25.0%)	-	-	-	-
White Race								
White	10	1.0 (10.0%)	6.0 (60.0%)	5.0 (50.0%)	6.00 (1.00, 35.91) 0.0497	13.50 (1.64, 111.19) 0.0156	50.0 (19.0, 81.0) 0.0016	
Other	25	1.3 (5.2%)	7.1 (28.4%)	10.0 (40.0%)	5.76 (0.74, 45.16) 0.0954	7.63 (0.80, 73.07) 0.0779	23.2 (2.1, 44.3) 0.0312	0.7172

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visis data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD where performed by imputation, and the results were pooled using Rubin's rules.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.1.2. Haem-A-QoL Physical Health Domain score response (≥ 15 points Improvement from baseline) analysis.

Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Overall								
-	35	7.9 (22.6%)	19.9 (56.9%)	18.0 (51.4%)	2.53 (1.18, 5.44) 0.0172	4.54 (1.41, 14.63) 0.0112	34.3 (10.4, 58.2) 0.0050	
Disease type								
Hemophilia A	30	7.9 (26.3%)	15.9 (53.0%)	13.0 (43.3%)	2.02 (0.94, 4.37) 0.0731	3.17 (0.95, 10.63) 0.0616	26.7 (0.3, 53.0) 0.0471	
Hemophilia B	5	0.0 (0.0%)	4.0 (80.0%)	5.0 (100.0%)	4.00 (0.73, 21.84) 0.1094	16.00 (1.09, 234.25) 0.0429	60.0 (17.1, 102.9) 0.0062	0.2811
Region								
Asia	18	2.0 (11.1%)	12.0 (66.7%)	11.0 (61.1%)	-	-	-	
Europe	5	1.0 (20.0%)	3.0 (60.0%)	3.0 (60.0%)	-	-	-	
Other	12	4.9 (40.8%)	4.9 (40.8%)	4.0 (33.3%)	-	-	-	-
White Race								
White	10	3.0 (30.0%)	6.0 (60.0%)	5.0 (50.0%)	2.00 (0.50, 8.00) 0.3270	3.50 (0.29, 41.99) 0.3231	30.0 (-25.8, 85.8) 0.2918	
Other	25	4.9 (19.6%)	13.9 (55.6%)	13.0 (52.0%)	2.88 (1.13, 7.30) 0.0262	5.21 (1.41, 19.19) 0.0133	36.0 (11.0, 61.0) 0.0047	0.7813

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visis data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD were performed by imputation, and the results were pooled using Rubin's rules.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
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 Inhibitor with On-Demand at OP
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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 9.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Dealing with Hemophilia										
Overall										
Modified Intent-to-Treat	35	4 (11.4%)	11 (31.4%)	11 (31.4%)	2.75	(0.95, 7.98)	3.55	(0.98, 12.92)	20.0 (0.9, 39.1)	
Set						0.0627		0.0544	0.0399	
Disease type										
Hemophilia A	30	4 (13.3%)	10 (33.3%)	9 (30.0%)	2.50	(0.85, 7.31)	3.25	(0.85, 12.41)	20.0 (-1.5, 41.5)	
						0.0943		0.0847	0.0679	
Hemophilia B	5	0 (0.0%)	1 (20.0%)	2 (40.0%)	2.00	(0.18, 22.06)	2.67	(0.09, 75.80)	20.0 (-45.6, 85.6)	0.9155
						0.5714		0.5658	0.5501	
Region										
Asia	18	3 (16.7%)	6 (33.3%)	6 (33.3%)		-		-	-	
Europe	5	1 (20.0%)	2 (40.0%)	2 (40.0%)		-		-	-	
Other	12	0 (0.0%)	3 (25.0%)	3 (25.0%)		-		-	-	-
White Race										
White	10	1 (10.0%)	2 (20.0%)	3 (30.0%)	2.00	(0.18, 22.06)	2.25	(0.14, 36.32)	10.0 (-23.4, 43.4)	
						0.5714		0.5677	0.5571	
Other	25	3 (12.0%)	9 (36.0%)	8 (32.0%)	3.00	(0.91, 9.89)	4.12	(0.95, 17.95)	24.0 (1.1, 46.9)	0.7224
						0.0710		0.0589	0.0403	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Family Planning										
Overall										
Modified Intent-to-Treat Set	34	5 (14.7%)	7 (20.6%)	6 (17.6%)	1.40 (0.49, 3.99) 0.5290		1.50 (0.42, 5.33) 0.5275		5.9 (-12.2, 24.0) 0.5246	
Disease type										
Hemophilia A	29	4 (13.8%)	6 (20.7%)	5 (17.2%)	1.50 (0.48, 4.65) 0.4825		1.63 (0.42, 6.33) 0.4800		6.9 (-12.1, 25.8) 0.4757	
Hemophilia B	5	1 (20.0%)	1 (20.0%)	1 (20.0%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.03, 31.97) 1.0000		0.0 (-55.4, 55.4) 1.0000	0.7997
Region										
Asia	17	2 (11.8%)	2 (11.8%)	2 (11.8%)	-		-		-	
Europe	5	2 (40.0%)	2 (40.0%)	2 (40.0%)	-		-		-	
Other	12	1 (8.3%)	3 (25.0%)	2 (16.7%)	-		-		-	-
White Race										
White	10	2 (20.0%)	3 (30.0%)	3 (30.0%)	1.50 (0.38, 6.00) 0.5664		1.71 (0.28, 10.59) 0.5618		10.0 (-23.4, 43.4) 0.5571	
Other	24	3 (12.5%)	4 (16.7%)	3 (12.5%)	1.33 (0.30, 5.96) 0.7064		1.40 (0.24, 8.04) 0.7059		4.2 (-17.4, 25.7) 0.7046	0.8750

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

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[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Feeling										
Overall										
Modified Intent-to-Treat Set	35	9 (25.7%)	16 (45.7%)	16 (45.7%)	1.78 (0.94, 3.35) 0.0746		2.43 (0.94, 6.28) 0.0662		20.0 (-0.7, 40.7) 0.0577	
Disease type										
Hemophilia A	30	8 (26.7%)	13 (43.3%)	12 (40.0%)	1.62 (0.81, 3.25) 0.1697		2.10 (0.74, 5.95) 0.1612		16.7 (-6.1, 39.5) 0.1517	
Hemophilia B	5	1 (20.0%)	3 (60.0%)	4 (80.0%)	3.00 (0.61, 14.86) 0.1785		6.00 (0.67, 53.68) 0.1090		40.0 (-2.9, 82.9) 0.0679	0.3919
Region										
Asia	18	6 (33.3%)	8 (44.4%)	8 (44.4%)	-		-		-	
Europe	5	2 (40.0%)	5 (100.0%)	4 (80.0%)	-		-		-	
Other	12	1 (8.3%)	3 (25.0%)	4 (33.3%)	-		-		-	-
White Race										
White	10	2 (20.0%)	6 (60.0%)	6 (60.0%)	3.00 (0.97, 9.30) 0.0571		6.00 (1.27, 28.25) 0.0234		40.0 (9.6, 70.4) 0.0098	
Other	25	7 (28.0%)	10 (40.0%)	10 (40.0%)	1.43 (0.66, 3.11) 0.3682		1.71 (0.54, 5.48) 0.3635		12.0 (-13.6, 37.6) 0.3577	0.2085

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 4 of 9.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
DOMAIN: Future								
Overall								
Modified Intent-to-Treat Set	35	5 (14.3%)	13 (37.1%)	15 (42.9%)	2.60 (1.05, 6.46) 0.0395	3.55 (1.12, 11.26) 0.0318	22.9 (3.3, 42.4) 0.0218	
Disease type								
Hemophilia A	30	5 (16.7%)	11 (36.7%)	12 (40.0%)	2.20 (0.88, 5.50) 0.0914	2.89 (0.88, 9.57) 0.0814	20.0 (-1.5, 41.5) 0.0679	
Hemophilia B	5	0 (0.0%)	2 (40.0%)	3 (60.0%)	3.00 (0.31, 28.84) 0.3414	6.00 (0.17, 214.89) 0.3264	40.0 (-30.1, 110.1) 0.2636	0.6919
Region								
Asia	18	1 (5.6%)	6 (33.3%)	7 (38.9%)	-	-	-	
Europe	5	2 (40.0%)	4 (80.0%)	2 (40.0%)	-	-	-	
Other	12	2 (16.7%)	3 (25.0%)	6 (50.0%)	-	-	-	-
White Race								
White	10	3 (30.0%)	5 (50.0%)	4 (40.0%)	1.67 (0.48, 5.76) 0.4193	2.33 (0.31, 17.66) 0.4120	20.0 (-26.4, 66.4) 0.3980	
Other	25	2 (8.0%)	8 (32.0%)	11 (44.0%)	4.00 (1.00, 15.99) 0.0499	5.41 (1.11, 26.45) 0.0370	24.0 (3.9, 44.1) 0.0191	0.5278

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

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[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Partnership and Sexuality										
Overall										
Modified Intent-to-Treat Set	35	11 (31.4%)	11 (31.4%)	10 (28.6%)	1.00	(0.49, 2.04) 1.0000	1.00	(0.35, 2.83) 1.0000	0.0	(-22.4, 22.4) 1.0000
Disease type										
Hemophilia A	30	9 (30.0%)	11 (36.7%)	9 (30.0%)	1.22	(0.58, 2.55) 0.5936	1.35	(0.45, 4.07) 0.5926	6.7	(-17.7, 31.0) 0.5912
Hemophilia B	5	2 (40.0%)	0 (0.0%)	1 (20.0%)	0.33	(0.03, 3.20) 0.3414	0.17	(0.00, 5.97) 0.3264	-40.0	(-110.1, 30.1) 0.2636
Region										
Asia	18	6 (33.3%)	5 (27.8%)	3 (16.7%)	-		-		-	
Europe	5	3 (60.0%)	3 (60.0%)	4 (80.0%)	-		-		-	
Other	12	2 (16.7%)	3 (25.0%)	3 (25.0%)	-		-		-	
White Race										
White	10	4 (40.0%)	4 (40.0%)	6 (60.0%)	1.00	(0.30, 3.32) 1.0000	1.00	(0.14, 7.39) 1.0000	0.0	(-48.0, 48.0) 1.0000
Other	25	7 (28.0%)	7 (28.0%)	4 (16.0%)	1.00	(0.41, 2.42) 1.0000	1.00	(0.29, 3.42) 1.0000	-0.0	(-24.8, 24.8) 1.0000

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Sport and Leisure										
Overall										
Modified Intent-to-Treat Set	35	3 (8.6%)	8 (22.9%)	7 (20.0%)	2.67 (0.80, 8.86) 0.1092		3.16 (0.80, 12.45) 0.1000		14.3 (-1.8, 30.4) 0.0824	
Disease type										
Hemophilia A	30	3 (10.0%)	6 (20.0%)	5 (16.7%)	2.00 (0.59, 6.79) 0.2663		2.25 (0.55, 9.21) 0.2594		10.0 (-6.9, 26.9) 0.2465	
Hemophilia B	5	0 (0.0%)	2 (40.0%)	2 (40.0%)	2.00 (0.18, 22.06) 0.5714		2.67 (0.09, 75.80) 0.5658		20.0 (-45.6, 85.6) 0.5501	
Region										
Asia	18	1 (5.6%)	4 (22.2%)	3 (16.7%)	-		-		-	
Europe	5	1 (20.0%)	1 (20.0%)	0 (0.0%)	-		-		-	
Other	12	1 (8.3%)	3 (25.0%)	4 (33.3%)	-		-		-	
White Race										
White	10	1 (10.0%)	2 (20.0%)	1 (10.0%)	2.00 (0.50, 8.00) 0.3270		2.25 (0.48, 10.60) 0.3050		10.0 (-8.6, 28.6) 0.2918	
Other	25	2 (8.0%)	6 (24.0%)	6 (24.0%)	3.00 (0.61, 14.86) 0.1785		3.63 (0.58, 22.77) 0.1685		16.0 (-5.3, 37.3) 0.1404	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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Event	N	Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
DOMAIN: Treatment								
Overall								
Set Modified Intent-to-Treat	35	7 (20.0%)	15 (42.9%)	18 (51.4%)	2.14 (1.05, 4.38) 0.0369	3.00 (1.12, 8.05) 0.0291	22.9 (3.3, 42.4) 0.0218	
Disease type								
Hemophilia A	30	7 (23.3%)	12 (40.0%)	13 (43.3%)	1.71 (0.84, 3.48) 0.1364	2.19 (0.80, 5.99) 0.1263	16.7 (-4.2, 37.5) 0.1169	
Hemophilia B	5	0 (0.0%)	3 (60.0%)	5 (100.0%)	NE	NE	NE	-
Region								
Asia	18	2 (11.1%)	9 (50.0%)	10 (55.6%)	-	-	-	
Europe	5	3 (60.0%)	4 (80.0%)	3 (60.0%)	-	-	-	
Other	12	2 (16.7%)	2 (16.7%)	5 (41.7%)	-	-	-	-
White Race								
White	10	4 (40.0%)	5 (50.0%)	6 (60.0%)	1.25 (0.47, 3.33) 0.6554	1.50 (0.26, 8.82) 0.6537	10.0 (-33.4, 53.4) 0.6514	
Other	25	3 (12.0%)	10 (40.0%)	12 (48.0%)	3.33 (1.14, 9.75) 0.0279	4.89 (1.32, 18.06) 0.0173	28.0 (7.2, 48.8) 0.0083	0.3077

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: View of Yourself										
Overall										
Modified Intent-to-Treat Set	35	7 (20.0%)	13 (37.1%)	13 (37.1%)	1.86 (0.86, 4.01) 0.1145		2.36 (0.83, 6.71) 0.1062		17.1 (-3.0, 37.3) 0.0957	
Disease type										
Hemophilia A	30	7 (23.3%)	10 (33.3%)	9 (30.0%)	1.43 (0.66, 3.11) 0.3682		1.64 (0.56, 4.80) 0.3640		10.0 (-11.4, 31.4) 0.3591	
Hemophilia B	5	0 (0.0%)	3 (60.0%)	4 (80.0%)	3.00 (0.31, 28.84) 0.3414		6.00 (0.17, 214.89) 0.3264		40.0 (-30.1, 110.1) 0.2636	0.4727
Region										
Asia	18	4 (22.2%)	8 (44.4%)	7 (38.9%)	-		-		-	
Europe	5	2 (40.0%)	2 (40.0%)	2 (40.0%)	-		-		-	
Other	12	1 (8.3%)	3 (25.0%)	4 (33.3%)	-		-		-	-
White Race										
White	10	2 (20.0%)	2 (20.0%)	4 (40.0%)	1.00 (0.14, 7.10) 1.0000		1.00 (0.09, 11.59) 1.0000		0.0 (-39.2, 39.2) 1.0000	
Other	25	5 (20.0%)	11 (44.0%)	9 (36.0%)	2.20 (0.95, 5.07) 0.0644		3.14 (0.99, 9.98) 0.0520		24.0 (1.1, 46.9) 0.0403	0.4380

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

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[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.1. Haem-A-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

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		Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)				
DOMAIN: Work and School								
Overall								
Modified Intent-to-Treat Set	33	2 (6.1%)	15 (45.5%)	14 (42.4%)	7.50 (2.06, 27.25) 0.0022	12.92 (3.18, 52.55) 0.0004	39.4 (22.7, 56.1) <.0001	
Disease type								
Hemophilia A	29	2 (6.9%)	12 (41.4%)	11 (37.9%)	6.00 (1.69, 21.26) 0.0055	9.53 (2.38, 38.11) 0.0014	34.5 (17.2, 51.8) <.0001	
Hemophilia B	4	0 (0.0%)	3 (75.0%)	3 (75.0%)	3.00 (0.61, 14.86) 0.1785	9.00 (0.66, 122.79) 0.0994	50.0 (1.0, 99.0) 0.0455	0.9699
Region								
Asia	18	1 (5.6%)	10 (55.6%)	9 (50.0%)	-	-	-	
Europe	5	1 (20.0%)	3 (60.0%)	3 (60.0%)	-	-	-	
Other	10	0 (0.0%)	2 (20.0%)	2 (20.0%)	-	-	-	-
White Race								
White	9	1 (11.1%)	4 (44.4%)	4 (44.4%)	-	-	-	
Other	24	1 (4.2%)	11 (45.8%)	10 (41.7%)	-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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8.1.2 Sensitivitätsanalyse

Pfizer Inc.: Final

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Inhibitor with On-Demand at OP

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TABLE 6.1.1. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Total Score.

Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 50.3 (18.16) 37.2 51.15 64.8	-	-
OP Day 180	n=32 52.5 (19.12) 38.6 49.99 63.2	n=31 0.8 (11.48) -4.9 1.38 10.1	1.5 (-2.4, 5.5)
ATP Baseline	n=35 51.3 (18.96) 37.0 51.22 64.7	-	-
ATP Day 180	n=32 38.4 (18.41) 23.9 36.41 49.4	n=32 -13.2 (17.47) -22.1 -11.41 -0.6	-12.0 (-18.3, -5.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-13.5 (-19.8, -7.2) <.0001
ATP Day 360	n=34 38.8 (17.05) 27.2 35.42 48.3	n=34 -12.8 (20.98) -19.7 -8.79 2.5	-11.0 (-17.6, -4.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.1. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Total Score
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 49.7 (18.89) 37.0 50.56 65.2	-	-
OP Day 180	n=27 52.1 (20.37) 38.6 48.65 64.4	n=27 0.9 (12.28) -8.5 1.38 10.7	1.6 (-3.8, 7.1)
ATP Baseline	n=30 50.7 (20.03) 37.0 50.61 62.2	-	-
ATP Day 180	n=28 38.9 (19.21) 23.7 36.41 49.4	n=28 -12.8 (18.42) -22.1 -8.16 -0.3	-10.7 (-19.1, -2.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-12.2 (-20.2, -4.3)
ATP Day 360	n=29 40.2 (17.78) 27.2 36.41 50.0	n=29 -10.8 (21.63) -16.4 -5.98 4.3	0.0064 -8.0 (-16.3, 0.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.1. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Total Score
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 54.9 (12.08) 47.2 59.52 62.6	-	-
OP Day 180	n=5 54.9 (11.35) 54.3 57.74 58.5	n=4 0.1 (3.42) -2.5 0.29 2.6	-0.1 (-4.7, 4.4)
ATP Baseline	n=5 54.7 (11.45) 49.5 53.33 64.7	-	-
ATP Day 180	n=4 35.3 (12.88) 25.8 32.61 44.8	n=4 -16.3 (9.12) -21.2 -12.23 -11.4	-20.7 (-32.0, -9.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-18.9 (-31.4, -6.3)
ATP Day 360	n=5 30.5 (9.15) 25.5 29.17 29.3	n=5 -24.2 (12.84) -24.2 -23.91 -18.7	0.0073 -23.9 (-43.8, -4.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.2. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Dealing with Hemophilia.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 28.9 (20.23) 16.7 25.00 41.7	-	-
OP Day 180	n=32 33.1 (16.05) 20.8 29.17 41.7	n=31 3.5 (17.05) 0.0 0.00 16.7	6.3 (-8.3, 16.7)
ATP Baseline	n=35 31.2 (20.14) 16.7 33.33 41.7	-	-
ATP Day 180	n=32 29.0 (21.83) 16.7 25.00 41.7	n=32 -1.7 (27.19) -16.7 0.00 8.3	-6.3 (-14.6, 4.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-11.5 (-22.9, 4.2)
ATP Day 360	n=34 34.1 (32.20) 8.3 29.17 41.7	n=34 2.9 (29.65) -16.7 4.17 16.7	0.1112 0.0 (-12.5, 16.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.2. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Dealing with Hemophilia
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 29.7 (21.41) 16.7 29.17 50.0	-	-
OP Day 180	n=27 34.6 (16.94) 16.7 33.33 41.7	n=27 3.7 (18.25) 0.0 0.00 16.7	8.5 (-5.4, 22.4)
ATP Baseline	n=30 33.1 (20.82) 16.7 33.33 41.7	-	-
ATP Day 180	n=28 30.5 (22.85) 16.7 29.17 41.7	n=28 -1.9 (28.50) -16.7 -4.17 8.3	-4.2 (-17.3, 9.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-9.3 (-25.3, 6.8)
ATP Day 360	n=29 37.4 (33.68) 8.3 33.33 58.3	n=29 4.3 (31.31) -16.7 8.33 16.7	0.2276 1.1 (-15.6, 17.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.2. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Dealing with Hemophilia
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 22.9 (4.17) 20.8 25.00 25.0	-	-
OP Day 180	n=5 25.0 (5.89) 25.0 25.00 25.0	n=4 2.1 (4.17) 0.0 0.00 4.2	5.2 (1.7, 8.8)
ATP Baseline	n=5 20.0 (11.18) 8.3 25.00 25.0	-	-
ATP Day 180	n=4 18.8 (7.98) 12.5 20.83 25.0	n=4 0.0 (18.00) -12.5 4.17 12.5	2.1 (-21.6, 25.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-0.0 (-27.4, 27.4) 1.0000
ATP Day 360	n=5 15.0 (9.13) 16.7 16.67 16.7	n=5 -5.0 (17.28) -16.7 -8.33 8.3	-4.2 (-27.9, 19.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.3. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Family Planning.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=34			
OP Baseline	n=28 35.0 (30.14) 3.1 37.50 53.1	-	-
OP Day 180	n=24 38.5 (32.84) 15.6 25.00 70.8	n=22 -0.2 (17.99) -12.5 -3.13 12.5	-0.5 (-11.5, 9.4)
ATP Baseline	n=30 34.0 (28.73) 12.5 32.29 43.8	-	-
ATP Day 180	n=22 29.5 (29.58) 0.0 25.00 43.8	n=21 -8.8 (26.41) -18.8 -6.25 6.3	-1.0 (-13.5, 8.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-0.5 (-15.6, 12.5)
ATP Day 360	n=24 27.8 (24.83) 3.1 25.00 50.0	n=23 -8.1 (20.62) -25.0 -6.25 8.3	0.8514 -4.7 (-21.9, 12.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.3. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Family Planning
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=29			
OP Baseline	n=25 34.0 (31.77) 0.0 25.00 56.3	-	-
OP Day 180	n=20 37.9 (33.25) 15.6 25.00 62.5	n=19 -2.2 (16.23) -12.5 -6.25 12.5	-0.8 (-12.0, 10.5)
ATP Baseline	n=25 32.2 (30.04) 0.0 25.00 43.8	-	-
ATP Day 180	n=18 26.0 (28.30) 0.0 21.88 43.8	n=17 -12.9 (26.23) -18.8 -6.25 0.0	-3.6 (-21.0, 13.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-3.0 (-20.4, 14.4)
ATP Day 360	n=20 26.9 (26.60) 0.0 18.75 50.0	n=19 -6.1 (20.11) -25.0 0.00 12.5	0.7062 -5.2 (-19.3, 8.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 Inhibitor with On-Demand at OP
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 TABLE 6.1.3. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Family Planning
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=3 43.1 (6.36) 37.5 41.67 50.0	-	-
OP Day 180	n=4 41.7 (35.36) 12.5 45.83 70.8	n=3 12.5 (27.32) -16.7 16.67 37.5	-1.5 (-45.9, 42.8)
ATP Baseline	n=5 42.9 (21.23) 31.3 37.50 62.5	-	-
ATP Day 180	n=4 45.3 (34.38) 18.8 53.13 71.9	n=4 8.3 (22.24) -5.2 6.25 21.9	6.3 (-24.5, 37.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	6.3 (-64.7, 77.2) 0.8484
ATP Day 360	n=4 32.3 (14.58) 20.8 31.25 43.8	n=4 -17.2 (23.59) -31.3 -12.50 -3.1	-12.5 (-51.6, 26.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.4. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Feeling.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 45.8 (25.69) 25.0 50.00 62.5	-	-
OP Day 180	n=32 44.1 (27.03) 28.1 43.75 62.5	n=31 -4.0 (18.93) -18.8 -6.25 6.3	-7.8 (-15.6, 3.1)
ATP Baseline	n=35 45.2 (29.78) 25.0 43.75 62.5	-	-
ATP Day 180	n=32 29.4 (29.30) 3.1 25.00 46.9	n=32 -15.3 (26.70) -37.5 -12.50 0.0	-16.4 (-28.1, -3.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-7.8 (-20.3, 6.2)
ATP Day 360	n=34 28.5 (27.06) 6.3 21.88 43.8	n=34 -17.1 (26.44) -31.3 -12.50 0.0	0.1723 -20.3 (-34.4, -6.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.4. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Feeling
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 44.4 (26.74) 25.0 50.00 62.5	-	-
OP Day 180	n=27 43.1 (28.97) 25.0 37.50 62.5	n=27 -3.5 (20.09) -18.8 -6.25 12.5	-3.0 (-13.7, 7.7)
ATP Baseline	n=30 44.4 (32.14) 25.0 40.63 62.5	-	-
ATP Day 180	n=28 30.7 (30.41) 3.1 25.00 50.0	n=28 -13.5 (27.36) -34.4 -6.25 3.1	-16.1 (-31.9, -0.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-9.3 (-25.3, 6.8)
ATP Day 360	n=29 29.1 (28.80) 0.0 25.00 43.8	n=29 -15.7 (27.83) -31.3 -6.25 0.0	0.2266 -18.8 (-34.1, -3.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.4. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Feeling
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 56.3 (13.50) 46.9 59.38 65.6	-	-
OP Day 180	n=5 50.0 (12.50) 43.8 56.25 56.3	n=4 -7.8 (7.86) -12.5 -6.25 -3.1	-8.3 (-17.2, 0.7)
ATP Baseline	n=5 50.0 (4.42) 50.0 50.00 50.0	-	-
ATP Day 180	n=4 20.3 (20.65) 3.1 18.75 37.5	n=4 -28.1 (19.43) -43.8 -28.13 -12.5	-32.5 (-60.8, -4.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-24.7 (-50.2, 0.9)
ATP Day 360	n=5 25.0 (14.66) 18.8 18.75 25.0	n=5 -25.0 (15.93) -31.3 -25.00 -25.0	0.0567 -29.7 (-40.3, -19.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.5. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Future.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 50.7 (25.14) 35.0 52.50 65.0	-	-
OP Day 180	n=32 50.8 (23.97) 37.5 45.00 67.5	n=31 -1.0 (21.35) -10.0 0.00 10.0	1.2 (-10.0, 10.0)
ATP Baseline	n=35 51.1 (24.83) 30.0 50.00 65.0	-	-
ATP Day 180	n=32 40.5 (22.37) 22.5 40.00 57.5	n=32 -9.8 (25.03) -25.0 -5.00 5.0	-8.8 (-17.5, 0.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-11.2 (-22.5, 2.5)
ATP Day 360	n=34 42.2 (23.20) 25.0 40.00 60.0	n=34 -9.3 (29.88) -25.0 -7.50 10.0	0.1019 -8.8 (-22.5, 5.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.5. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Future
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 49.2 (25.67) 30.0 50.00 65.0	-	-
OP Day 180	n=27 49.1 (25.15) 35.0 45.00 65.0	n=27 -0.9 (22.66) -10.0 0.00 10.0	3.1 (-9.5, 15.7)
ATP Baseline	n=30 49.8 (25.68) 30.0 50.00 65.0	-	-
ATP Day 180	n=28 40.4 (22.69) 22.5 40.00 57.5	n=28 -9.8 (26.19) -22.5 -5.00 5.0	-8.2 (-19.0, 2.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-12.3 (-27.3, 2.8) 0.0995
ATP Day 360	n=29 43.4 (23.98) 30.0 40.00 60.0	n=29 -6.7 (30.30) -25.0 -5.00 10.0	-5.8 (-18.0, 6.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.5. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Future
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 62.5 (19.36) 50.0 57.50 75.0	-	-
OP Day 180	n=5 60.0 (14.58) 50.0 55.00 70.0	n=4 -1.3 (10.31) -10.0 -2.50 7.5	-0.8 (-12.5, 11.0)
ATP Baseline	n=5 59.0 (19.17) 45.0 50.00 65.0	-	-
ATP Day 180	n=4 41.3 (23.23) 22.5 37.50 60.0	n=4 -10.0 (17.32) -25.0 -10.00 5.0	-23.7 (-56.4, 9.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-16.8 (-44.6, 11.1)
ATP Day 360	n=5 35.0 (18.37) 25.0 25.00 40.0	n=5 -24.0 (24.85) -25.0 -20.00 -10.0	0.2098 -23.8 (-55.0, 7.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.6. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Partnership and Sexuality.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 39.5 (33.54) 8.3 33.33 75.0	-	-
OP Day 180	n=32 38.8 (35.58) 4.2 33.33 70.8	n=31 -3.2 (32.25) -16.7 0.00 16.7	-4.2 (-20.8, 8.3)
ATP Baseline	n=35 40.2 (35.15) 0.0 33.33 66.7	-	-
ATP Day 180	n=32 35.2 (36.71) 0.0 25.00 62.5	n=32 -7.3 (41.19) -29.2 0.00 12.5	-12.5 (-29.2, 8.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-4.2 (-25.0, 16.7)
ATP Day 360	n=34 31.4 (33.15) 0.0 25.00 58.3	n=34 -9.1 (38.56) -25.0 0.00 8.3	0.6124 -8.3 (-33.3, 12.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.6. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Partnership and Sexuality
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 36.9 (33.09) 0.0 29.17 66.7	-	-
OP Day 180	n=27 40.4 (35.03) 8.3 33.33 75.0	n=27 0.0 (27.93) -16.7 0.00 16.7	0.7 (-15.8, 17.3)
ATP Baseline	n=30 41.7 (35.49) 0.0 33.33 66.7	-	-
ATP Day 180	n=28 34.2 (37.01) 0.0 25.00 50.0	n=28 -10.4 (42.35) -33.3 -4.17 4.2	-10.6 (-36.3, 15.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-8.8 (-34.1, 16.6)
ATP Day 360	n=29 32.2 (34.19) 0.0 25.00 58.3	n=29 -9.8 (40.83) -25.0 0.00 8.3	0.4593 -10.8 (-37.4, 15.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.6. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Partnership and Sexuality
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 58.3 (35.36) 29.2 54.17 87.5	-	-
OP Day 180	n=5 30.0 (41.50) 0.0 16.67 33.3	n=4 -25.0 (54.01) -62.5 -12.50 12.5	-33.8 (-104.9, 37.3)
ATP Baseline	n=5 31.7 (35.55) 0.0 25.00 50.0	-	-
ATP Day 180	n=4 41.7 (39.09) 8.3 45.83 75.0	n=4 14.6 (25.80) -4.2 8.33 33.3	4.6 (-47.2, 56.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	30.9 (-53.5, 115.2)
ATP Day 360	n=5 26.7 (29.11) 8.3 25.00 25.0	n=5 -5.0 (24.01) -8.3 0.00 0.0	0.4336 -8.3 (-46.2, 29.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.7. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Physical Health.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 55.9 (25.24) 35.0 55.00 75.0	-	-
OP Day 180	n=32 59.4 (25.33) 40.0 62.50 75.0	n=31 2.3 (19.44) -10.0 5.00 20.0	2.2 (-6.6, 11.0)
ATP Baseline	n=35 56.7 (25.29) 40.0 55.00 75.0	-	-
ATP Day 180	n=32 34.5 (27.52) 10.0 30.00 55.0	n=32 -22.7 (23.84) -32.5 -17.50 -5.0	-20.8 (-29.7, -11.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-25.9 (-37.5, -14.2) <.0001
ATP Day 360	n=34 36.6 (25.72) 15.0 32.50 55.0	n=34 -20.1 (25.33) -30.0 -12.50 -5.0	-20.9 (-31.1, -10.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.7. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Physical Health
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 55.5 (26.11) 35.0 52.50 75.0	-	-
OP Day 180	n=27 58.9 (27.15) 35.0 60.00 80.0	n=27 2.0 (20.72) -10.0 5.00 20.0	0.3 (-10.3, 11.0)
ATP Baseline	n=30 55.3 (26.29) 40.0 52.50 75.0	-	-
ATP Day 180	n=28 34.9 (28.42) 10.0 27.50 55.0	n=28 -22.1 (24.79) -30.0 -17.50 -5.0	-19.6 (-31.1, -8.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-23.5 (-38.3, -8.7)
ATP Day 360	n=29 36.6 (27.58) 15.0 30.00 55.0	n=29 -18.8 (26.58) -30.0 -10.00 -5.0	0.0054 -19.8 (-32.0, -7.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 Inhibitor with On-Demand at OP
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 TABLE 6.1.7. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Physical Health
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 58.8 (20.16) 45.0 65.00 72.5	-	-
OP Day 180	n=5 62.0 (13.04) 60.0 70.00 70.0	n=4 3.8 (7.50) -2.5 5.00 10.0	4.1 (-4.5, 12.6)
ATP Baseline	n=5 65.0 (18.03) 50.0 70.00 70.0	-	-
ATP Day 180	n=4 31.3 (23.23) 15.0 35.00 47.5	n=4 -27.5 (17.56) -42.5 -27.50 -12.5	-34.4 (-62.1, -6.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-35.0 (-57.0, -13.0)
ATP Day 360	n=5 37.0 (11.51) 30.0 35.00 40.0	n=5 -28.0 (16.05) -30.0 -20.00 -20.0	0.0054 -25.0 (-47.7, -2.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.8. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Sport and Leisure.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 72.0 (21.13) 60.0 77.50 90.0	-	-
OP Day 180	n=30 77.7 (21.62) 75.0 80.00 95.0	n=29 5.4 (25.21) -5.0 5.00 25.0	9.3 (-0.6, 16.0)
ATP Baseline	n=31 71.0 (24.09) 55.0 75.00 90.0	-	-
ATP Day 180	n=27 66.7 (24.16) 50.0 65.00 90.0	n=26 -4.4 (22.30) -18.8 0.00 0.0	-0.9 (-11.9, 10.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-7.4 (-17.4, 5.0)
ATP Day 360	n=28 65.4 (25.80) 48.1 67.50 86.3	n=27 -4.3 (26.52) -15.0 0.00 5.0	0.2661 0.2 (-7.5, 8.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.8. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Sport and Leisure
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 71.8 (20.67) 60.0 77.50 90.0	-	-
OP Day 180	n=25 75.7 (22.58) 75.0 80.00 95.0	n=25 4.3 (26.49) -5.0 5.00 25.0	5.6 (-5.4, 16.5)
ATP Baseline	n=26 68.3 (24.58) 55.0 67.50 87.5	-	-
ATP Day 180	n=23 67.7 (23.75) 50.0 65.00 90.0	n=22 -1.4 (20.02) -18.8 0.00 6.3	-1.9 (-16.3, 12.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-3.8 (-17.8, 10.2)
ATP Day 360	n=24 65.8 (26.47) 48.1 67.50 87.5	n=23 -1.6 (25.83) -12.5 0.00 10.0	0.5564 -5.7 (-20.5, 9.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.8. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Sport and Leisure
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 73.8 (27.80) 55.0 80.00 92.5	-	-
OP Day 180	n=5 88.0 (13.04) 90.0 95.00 95.0	n=4 12.5 (15.55) 0.0 12.50 25.0	13.8 (-6.1, 33.7)
ATP Baseline	n=5 85.0 (16.96) 90.0 90.00 95.0	-	-
ATP Day 180	n=4 61.3 (29.55) 37.5 65.00 85.0	n=4 -21.3 (29.83) -40.0 -10.00 -2.5	-20.1 (-54.2, 14.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-35.0 (-87.7, 17.7)
ATP Day 360	n=4 63.1 (24.78) 45.0 67.50 81.3	n=4 -19.4 (29.04) -40.0 -11.25 1.2	0.1694 -20.0 (-56.9, 16.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.9. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Treatment.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 51.5 (18.26) 37.5 56.25 62.5	-	-
OP Day 180	n=32 54.8 (20.70) 37.5 53.13 71.9	n=31 2.4 (17.39) -12.5 0.00 18.8	2.3 (-6.2, 10.9)
ATP Baseline	n=35 52.9 (21.30) 37.5 50.00 65.6	-	-
ATP Day 180	n=32 37.0 (24.14) 14.1 35.94 57.8	n=32 -16.7 (25.66) -32.8 -12.50 0.0	-21.1 (-31.2, -10.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-18.0 (-29.7, -9.4) 0.0001
ATP Day 360	n=34 36.4 (22.79) 15.6 34.38 50.0	n=34 -16.9 (28.14) -31.3 -15.63 3.1	-19.5 (-31.2, -7.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.9. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Treatment
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 51.9 (18.83) 37.5 56.25 65.6	-	-
OP Day 180	n=27 55.2 (21.49) 37.5 53.13 75.0	n=27 2.7 (18.46) -15.6 0.00 18.8	3.6 (-5.1, 12.3)
ATP Baseline	n=30 53.1 (22.64) 37.5 50.00 75.0	-	-
ATP Day 180	n=28 37.9 (24.26) 12.5 39.06 57.8	n=28 -15.6 (25.86) -29.7 -12.50 0.0	-18.5 (-30.5, -6.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-16.8 (-29.8, -3.8)
ATP Day 360	n=29 39.7 (22.84) 21.9 37.50 53.1	n=29 -13.9 (29.00) -28.1 -9.38 3.1	0.0166 -14.4 (-27.8, -1.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.9. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Treatment
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 48.4 (14.99) 35.9 50.00 60.9	-	-
OP Day 180	n=5 52.5 (17.59) 37.5 56.25 65.6	n=4 0.8 (8.22) -4.7 -1.56 6.3	5.1 (-8.3, 18.4)
ATP Baseline	n=5 51.9 (11.82) 40.6 59.38 59.4	-	-
ATP Day 180	n=4 30.5 (25.69) 15.6 18.75 45.3	n=4 -24.2 (26.44) -45.3 -29.69 -3.1	-18.8 (-50.7, 13.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-24.6 (-54.0, 4.7)
ATP Day 360	n=5 17.5 (10.50) 12.5 18.75 21.9	n=5 -34.4 (14.15) -40.6 -28.13 -25.0	0.0906 -32.8 (-52.4, -13.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.10. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - View of Yourself.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=35			
OP Baseline	n=34 55.9 (23.11) 40.0 60.00 70.0	-	-
OP Day 180	n=32 56.3 (22.97) 42.5 55.00 70.0	n=31 -2.4 (17.12) -10.0 0.00 10.0	-3.8 (-12.5, 5.0)
ATP Baseline	n=35 57.0 (22.10) 40.0 55.00 70.0	-	-
ATP Day 180	n=32 44.8 (22.59) 27.5 47.50 60.0	n=32 -13.3 (20.85) -27.5 -10.00 -2.5	-12.5 (-22.5, -5.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-11.2 (-22.5, 0.0) 0.0222
ATP Day 360	n=34 44.7 (21.35) 30.0 42.50 65.0	n=34 -12.6 (24.44) -25.0 -10.00 0.0	-11.2 (-22.5, -2.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

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TABLE 6.1.10. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - View of Yourself

Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=30			
OP Baseline	n=30 55.3 (24.35) 35.0 60.00 70.0	-	-
OP Day 180	n=27 54.3 (23.56) 40.0 50.00 70.0	n=27 -3.1 (18.14) -15.0 -5.00 10.0	-5.6 (-15.7, 4.5)
ATP Baseline	n=30 55.5 (22.87) 40.0 55.00 70.0	-	-
ATP Day 180	n=28 46.6 (21.60) 32.5 50.00 60.0	n=28 -10.9 (20.77) -22.5 -5.00 0.0	-9.5 (-18.2, -0.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-6.4 (-17.8, 5.1)
ATP Day 360	n=29 44.8 (21.94) 30.0 45.00 65.0	n=29 -11.0 (26.03) -15.0 -5.00 0.0	0.2438 -11.3 (-24.3, 1.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.10. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - View of Yourself
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=5			
OP Baseline	n=4 60.0 (10.80) 52.5 57.50 67.5	-	-
OP Day 180	n=5 67.0 (17.54) 60.0 60.00 85.0	n=4 2.5 (6.45) -2.5 2.50 7.5	8.5 (-8.0, 24.9)
ATP Baseline	n=5 66.0 (15.57) 55.0 60.00 80.0	-	-
ATP Day 180	n=4 32.5 (29.01) 15.0 22.50 50.0	n=4 -30.0 (13.54) -37.5 -35.00 -22.5	-35.0 (-56.3, -13.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-40.0 (-65.0, -15.0)
ATP Day 360	n=5 44.0 (19.81) 30.0 40.00 50.0	n=5 -22.0 (7.58) -25.0 -25.00 -20.0	0.0051 -22.5 (-33.9, -11.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.11. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Work and School.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=33			
OP Baseline	n=30 46.5 (22.18) 31.3 40.63 62.5	-	-
OP Day 180	n=30 50.6 (26.28) 31.3 46.88 68.8	n=27 4.3 (19.70) -6.3 0.00 18.8	5.7 (-7.3, 15.6)
ATP Baseline	n=33 52.4 (25.62) 37.5 50.00 68.8	-	-
ATP Day 180	n=31 33.4 (25.59) 18.8 25.00 56.3	n=31 -19.0 (26.20) -37.5 -12.50 0.0	-22.4 (-35.4, -9.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-22.4 (-37.5, -9.4)
ATP Day 360	n=31 34.7 (22.03) 12.5 37.50 50.0	n=31 -17.0 (29.65) -37.5 -12.50 6.3	0.0002 -17.2 (-34.4, -6.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.11. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Work and School
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=29			
OP Baseline	n=27 46.4 (22.88) 31.3 43.75 62.5	-	-
OP Day 180	n=26 52.2 (27.61) 31.3 50.00 75.0	n=24 5.6 (20.30) -6.3 0.00 21.9	5.9 (-7.9, 19.6)
ATP Baseline	n=29 53.6 (26.80) 37.5 50.00 75.0	-	-
ATP Day 180	n=27 34.4 (27.26) 16.7 25.00 56.3	n=27 -19.2 (28.01) -43.8 -6.25 0.0	-24.6 (-40.0, -9.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-22.2 (-37.3, -7.1)
ATP Day 360	n=27 37.6 (22.16) 18.8 50.00 50.0	n=27 -15.4 (30.80) -31.3 -6.25 6.3	0.0084 -18.1 (-34.4, -1.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.11. Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Work and School
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=4			
OP Baseline	n=3 47.9 (18.04) 37.5 37.50 68.8	-	-
OP Day 180	n=4 40.6 (13.01) 31.3 40.63 50.0	n=3 -6.3 (10.83) -12.5 -12.50 6.3	-10.9 (-21.6, -0.3)
ATP Baseline	n=4 43.8 (13.50) 34.4 46.88 53.1	-	-
ATP Day 180	n=4 26.6 (5.98) 21.9 28.13 31.3	n=4 -17.2 (7.86) -21.9 -18.75 -12.5	-18.8 (-29.4, -8.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-9.9 (-27.7, 7.9)
ATP Day 360	n=4 15.6 (6.25) 12.5 12.50 18.8	n=4 -28.1 (19.43) -40.6 -34.38 -15.6	0.2428 -37.5 (-44.6, -30.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

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Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 6.1.12. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Total Score.

Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 42.7 (14.81) 31.5 46.75 50.3	-	-
OP Day 180	n=12 42.7 (12.81) 33.3 43.34 53.7	n=12 -0.1 (11.74) -9.4 -2.60 9.1	-0.1 (-9.1, 9.0)
ATP Baseline	n=13 41.6 (12.67) 29.9 41.56 53.2	-	-
ATP Day 180	n=11 34.0 (11.60) 23.4 28.25 49.4	n=11 -8.6 (7.11) -15.3 -6.17 -3.9	-7.9 (-13.7, -2.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-8.3 (-18.2, 1.6) 0.1000
ATP Day 360	n=12 32.0 (13.40) 21.9 29.06 45.6	n=12 -10.7 (7.75) -15.7 -10.71 -5.0	-10.3 (-15.9, -4.6)

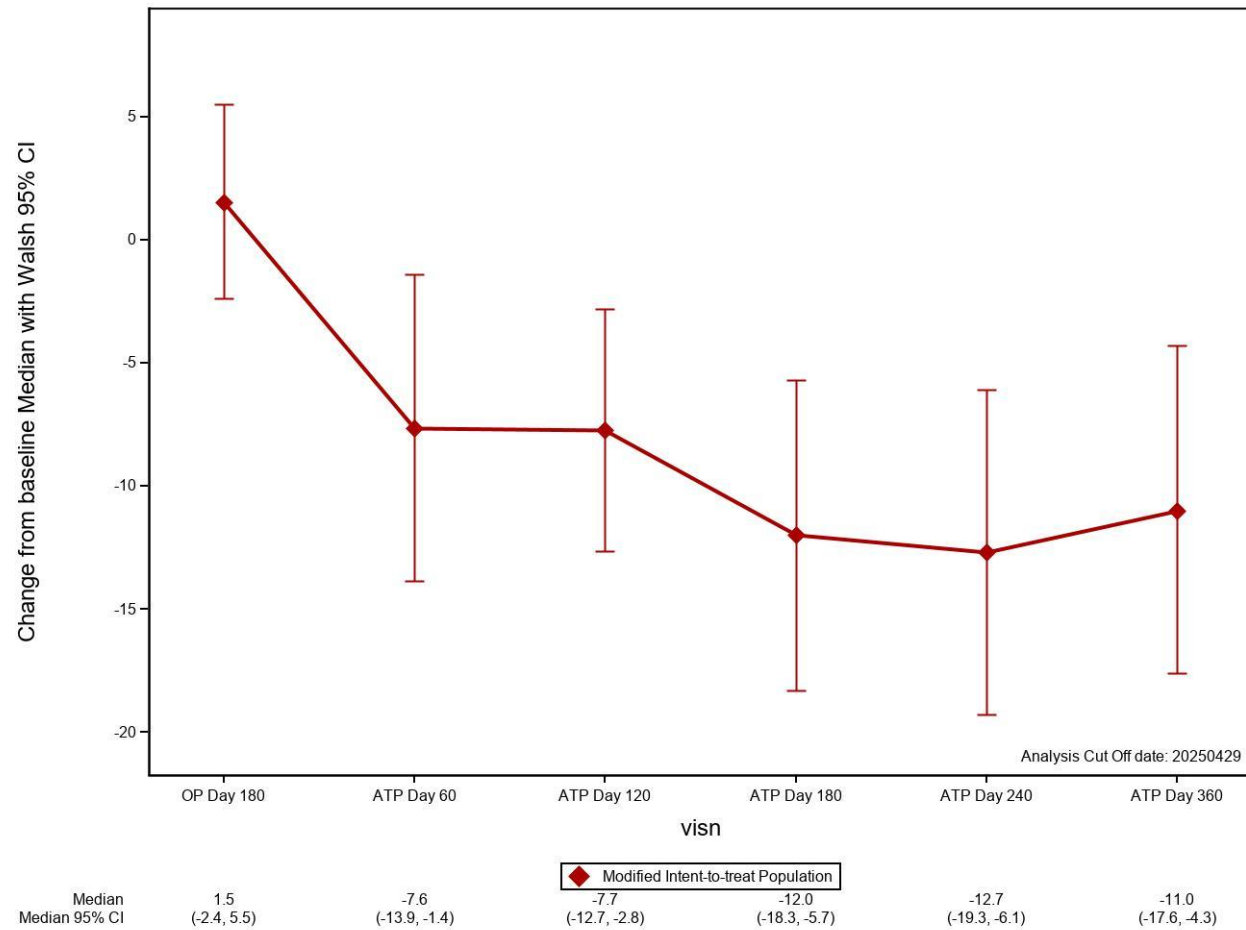
ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

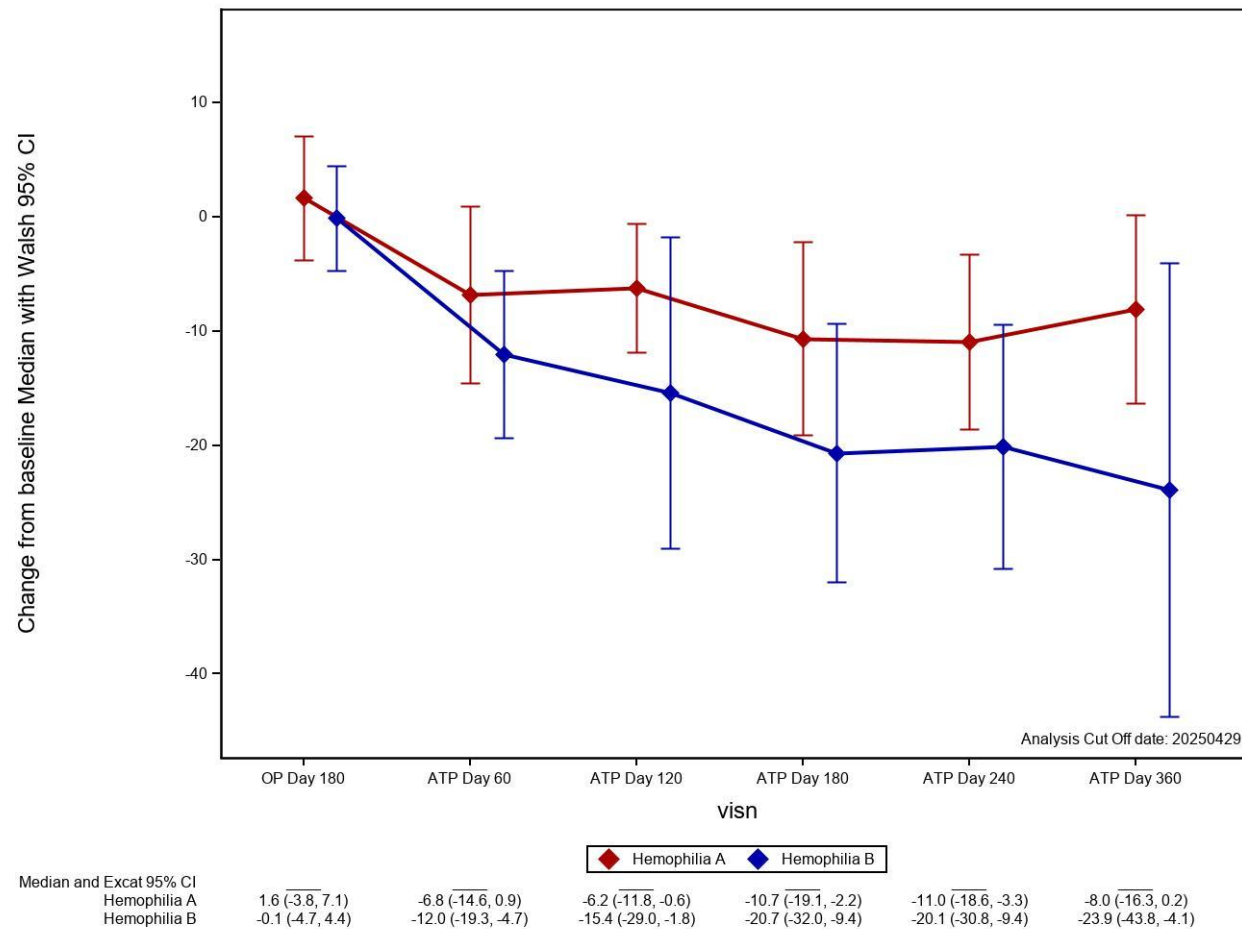
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.1.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Total Score – Modified Intent-to-Treat Set – Change from baseline Medians – Overall



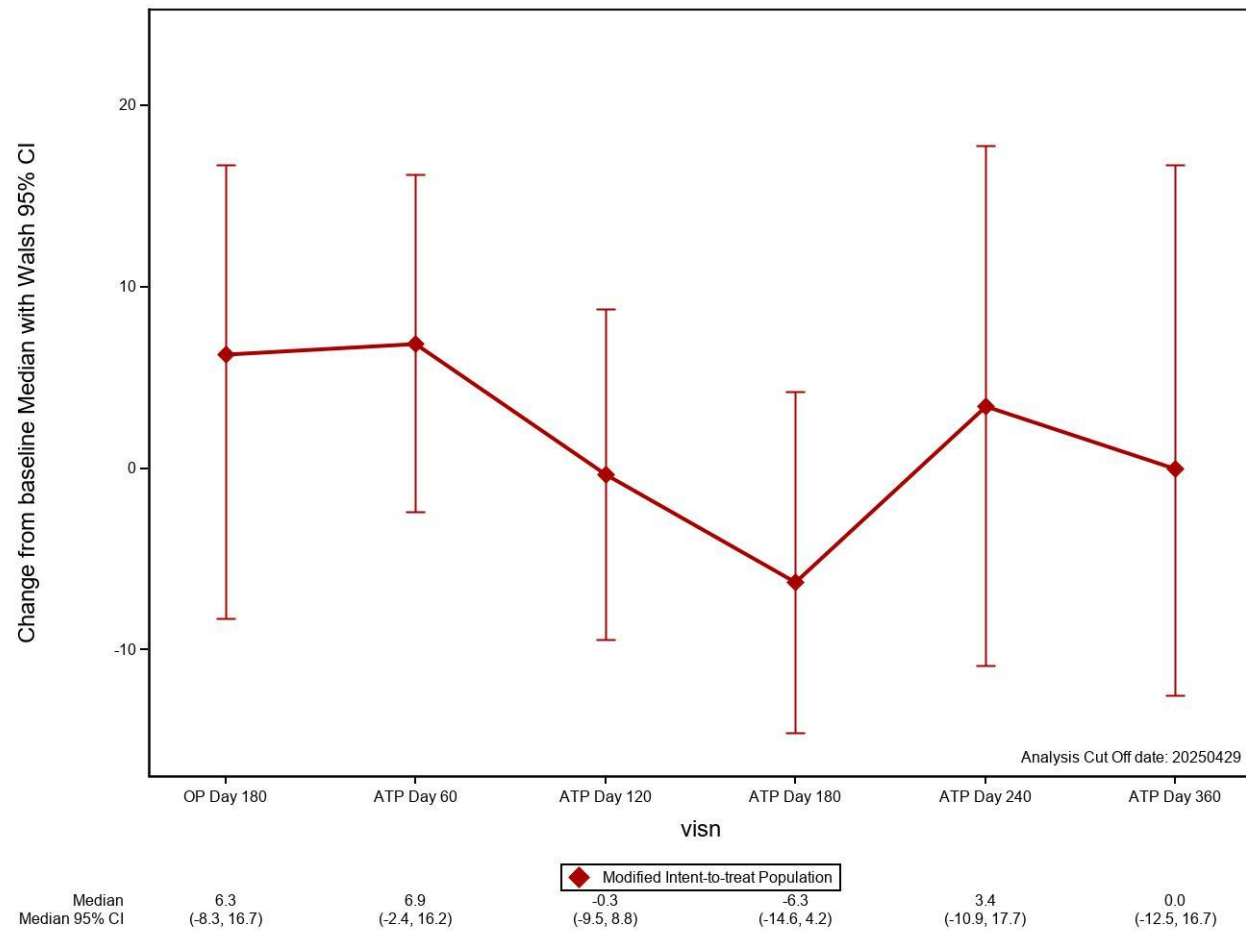
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.1.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Total Score – Modified Intent-to-Treat Set – Change from baseline Medians – By Disease Type



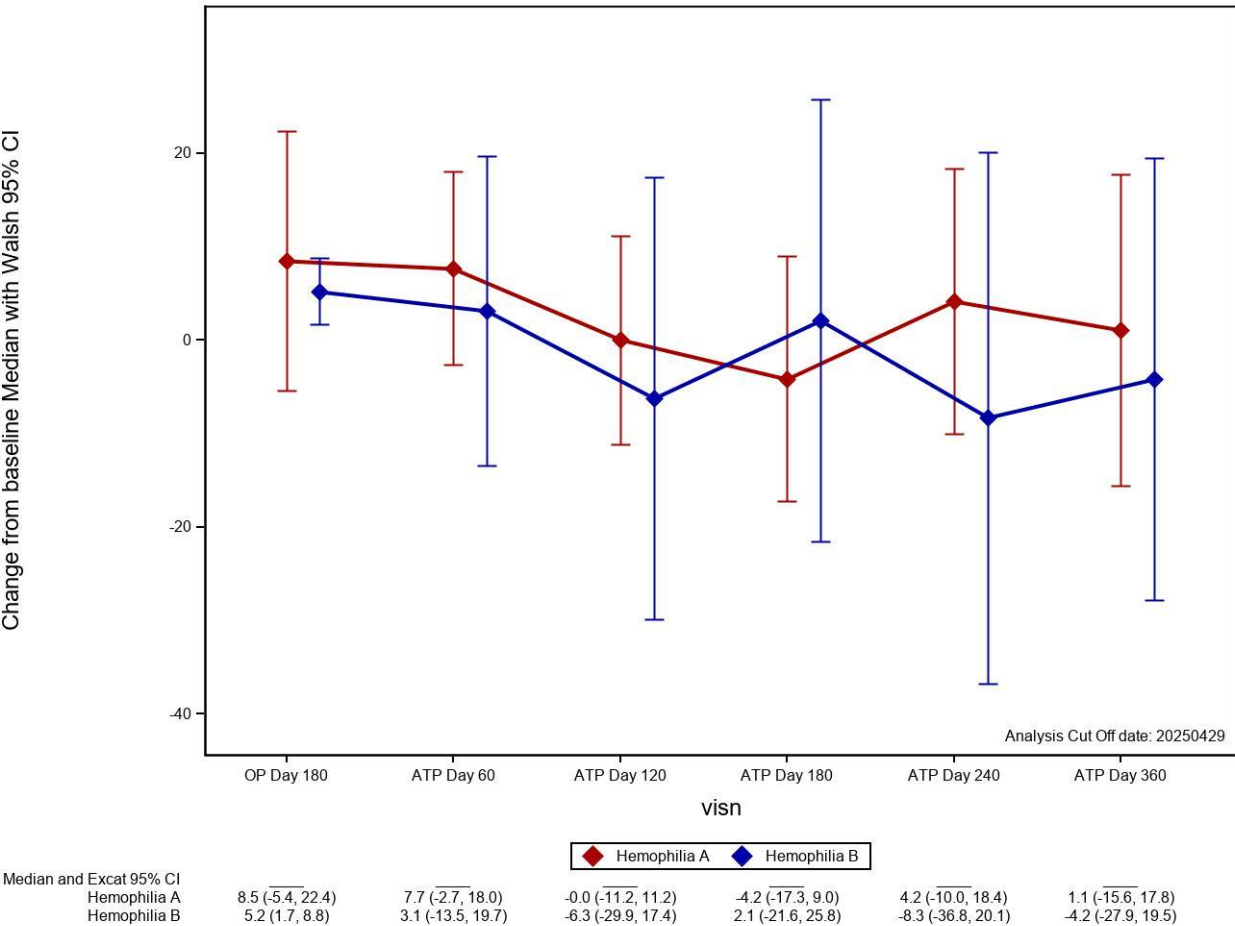
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.2.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Dealing with Hemophilia – Modified Intent-to-Treat Set – Change from baseline Medians – Overall



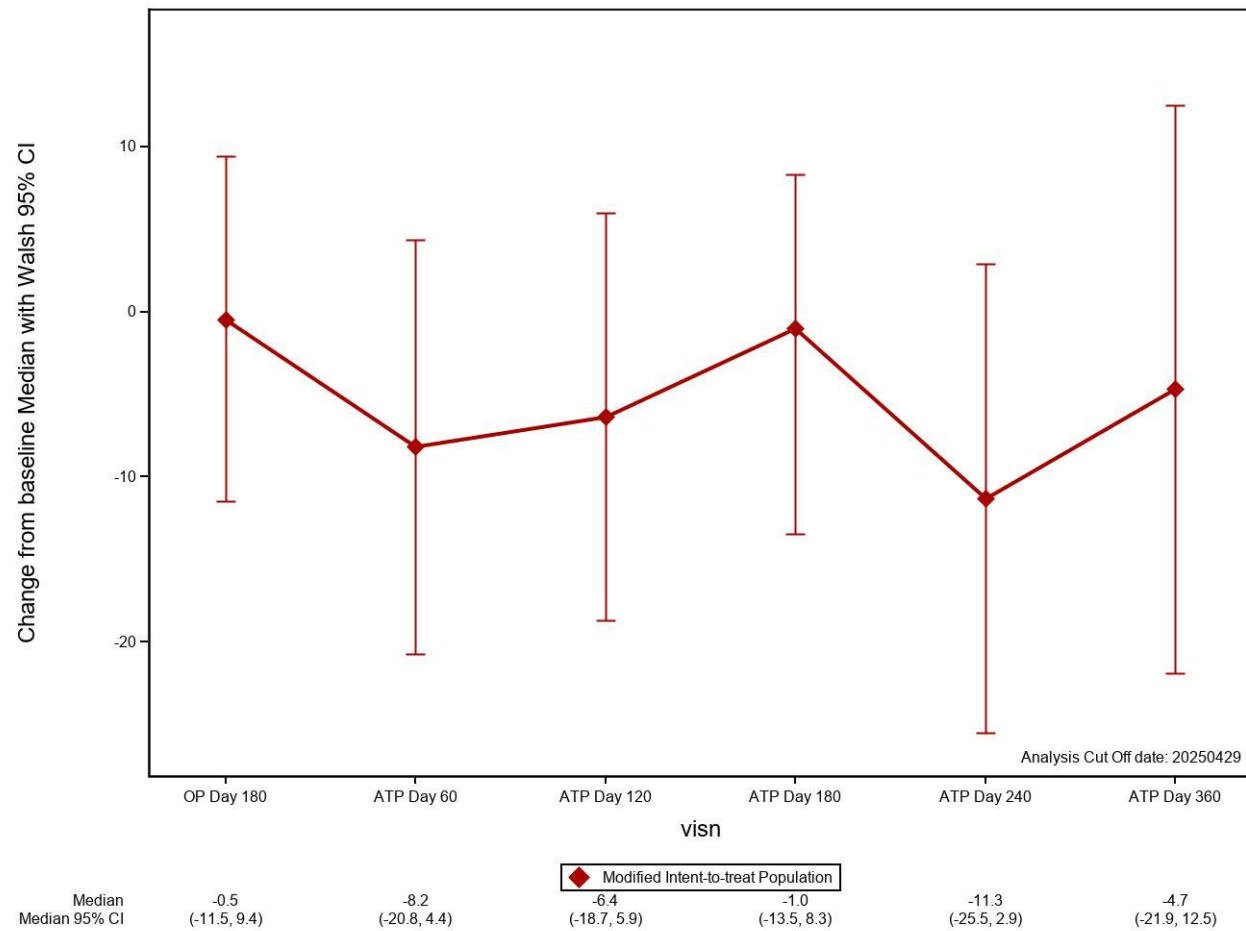
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.2.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Dealing with Hemophilia - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



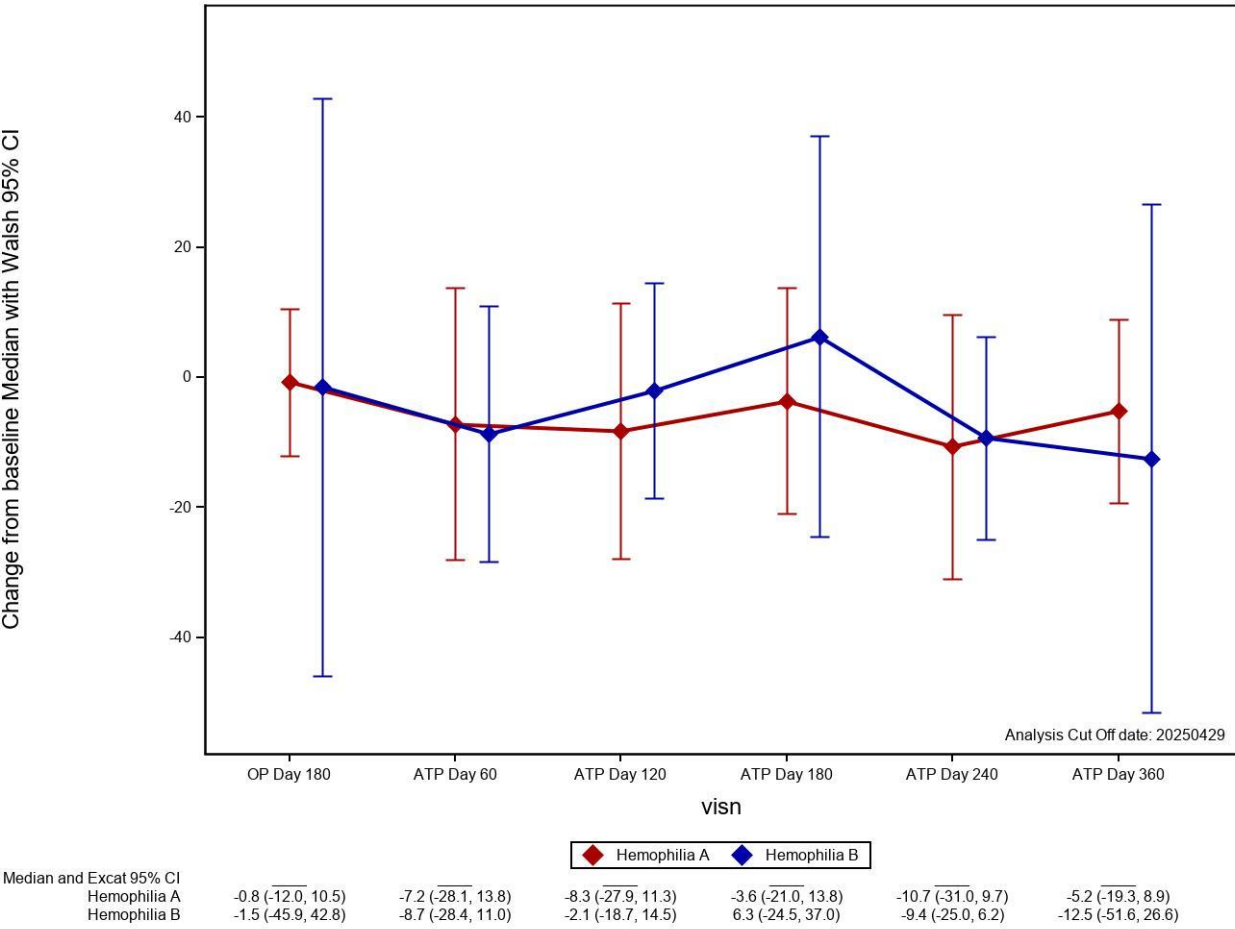
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.3.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Family Planning – Modified Intent-to-Treat Set – Change from baseline Medians – Overall



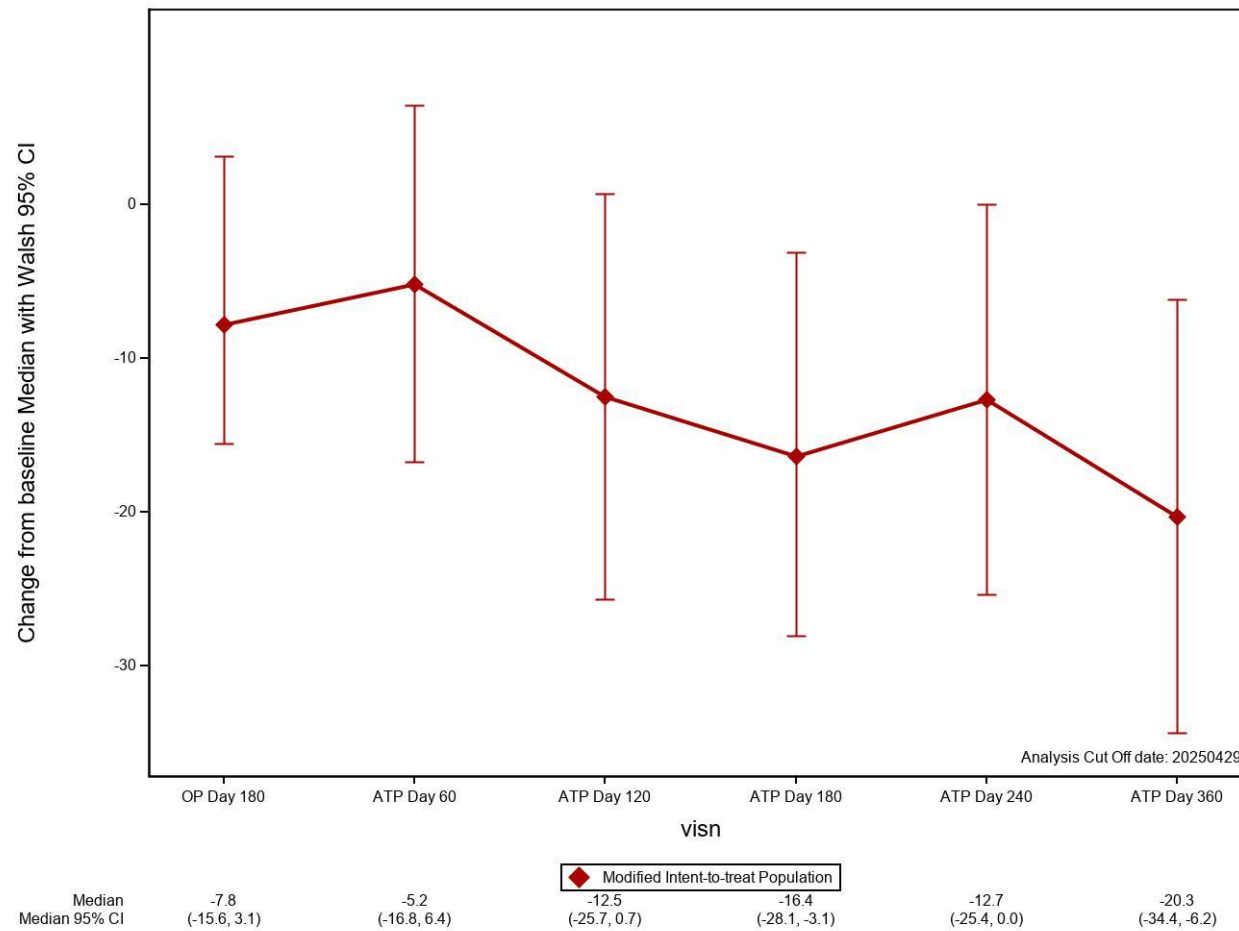
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.3.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Family Planning - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



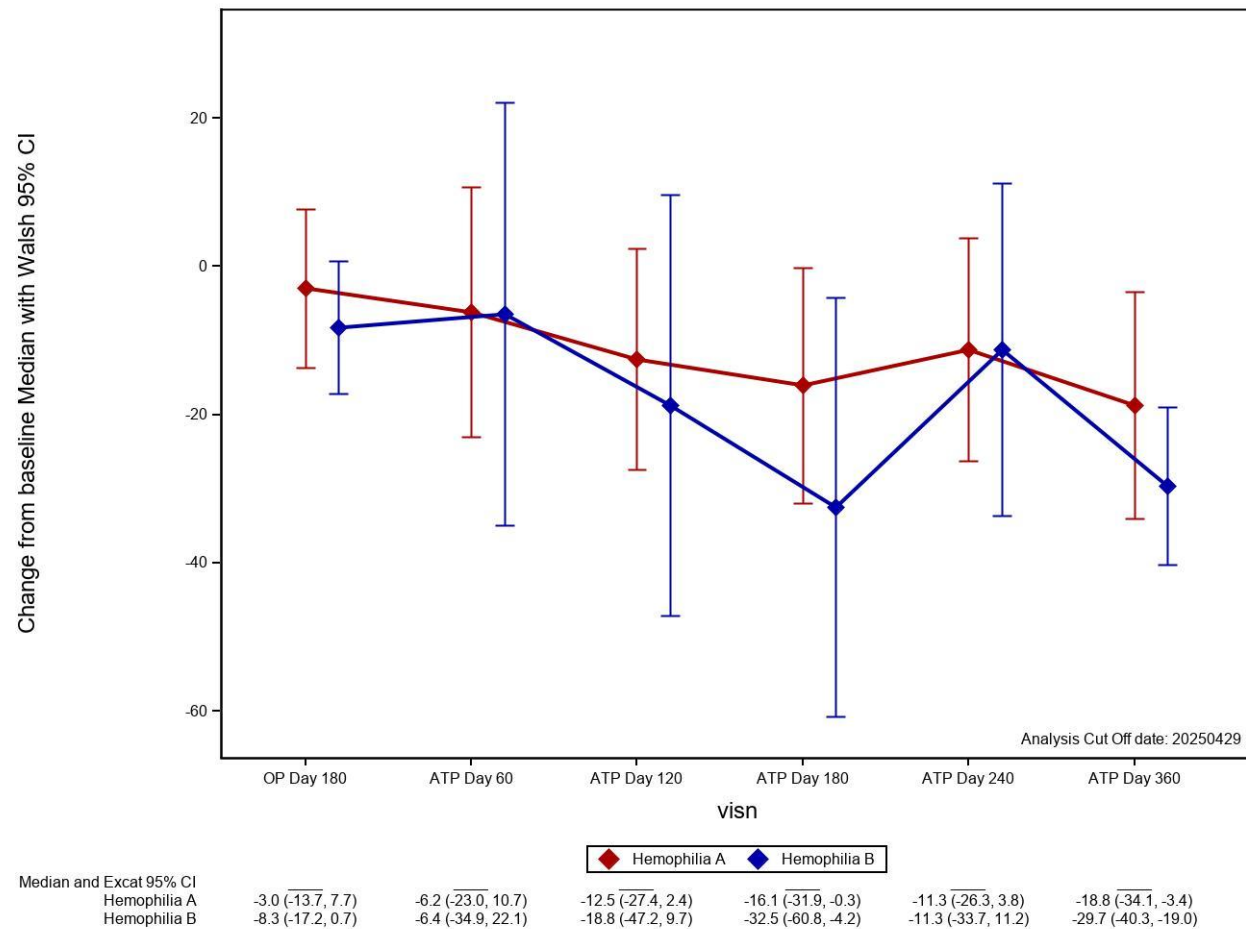
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.4.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Feeling - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



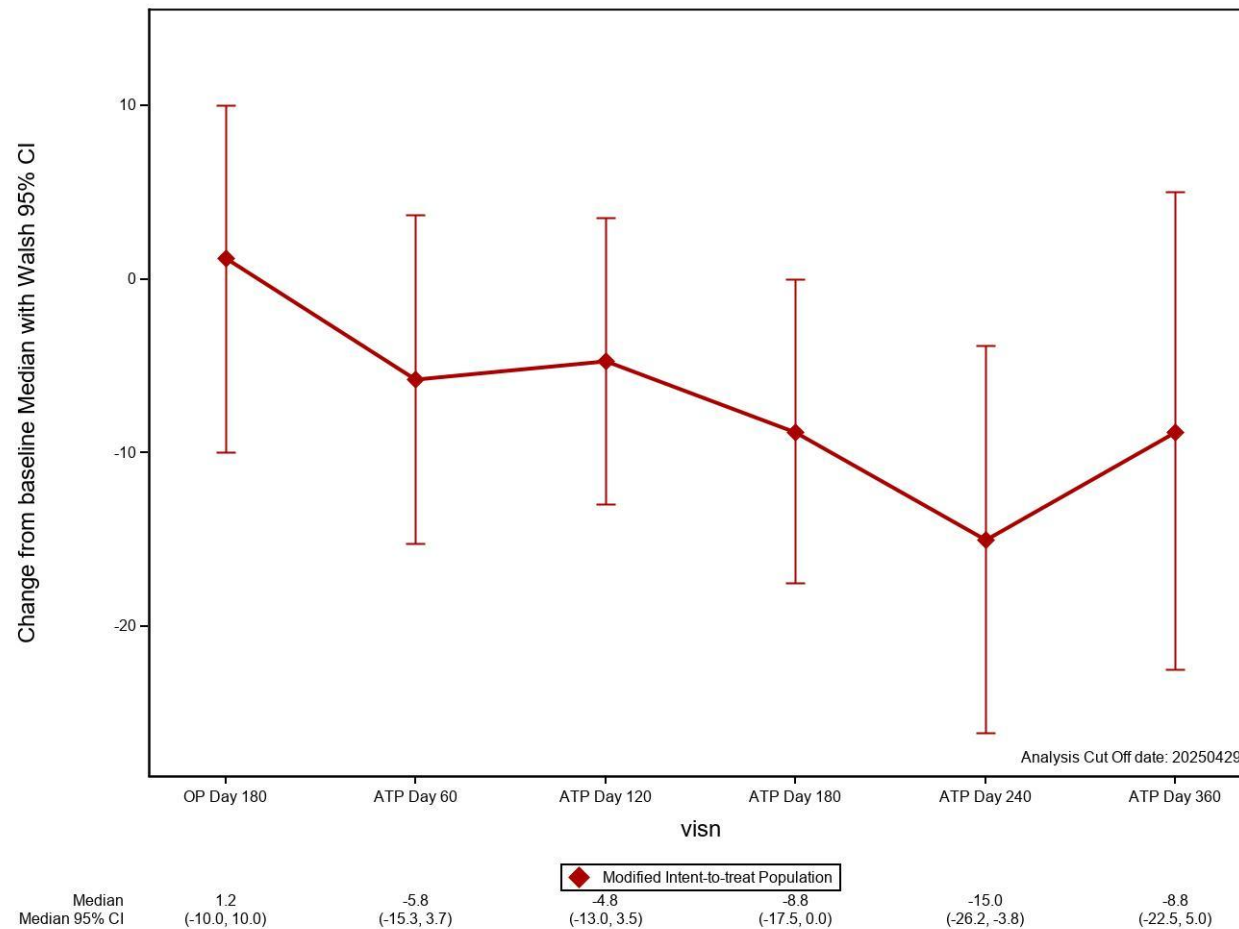
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.4.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Feeling - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



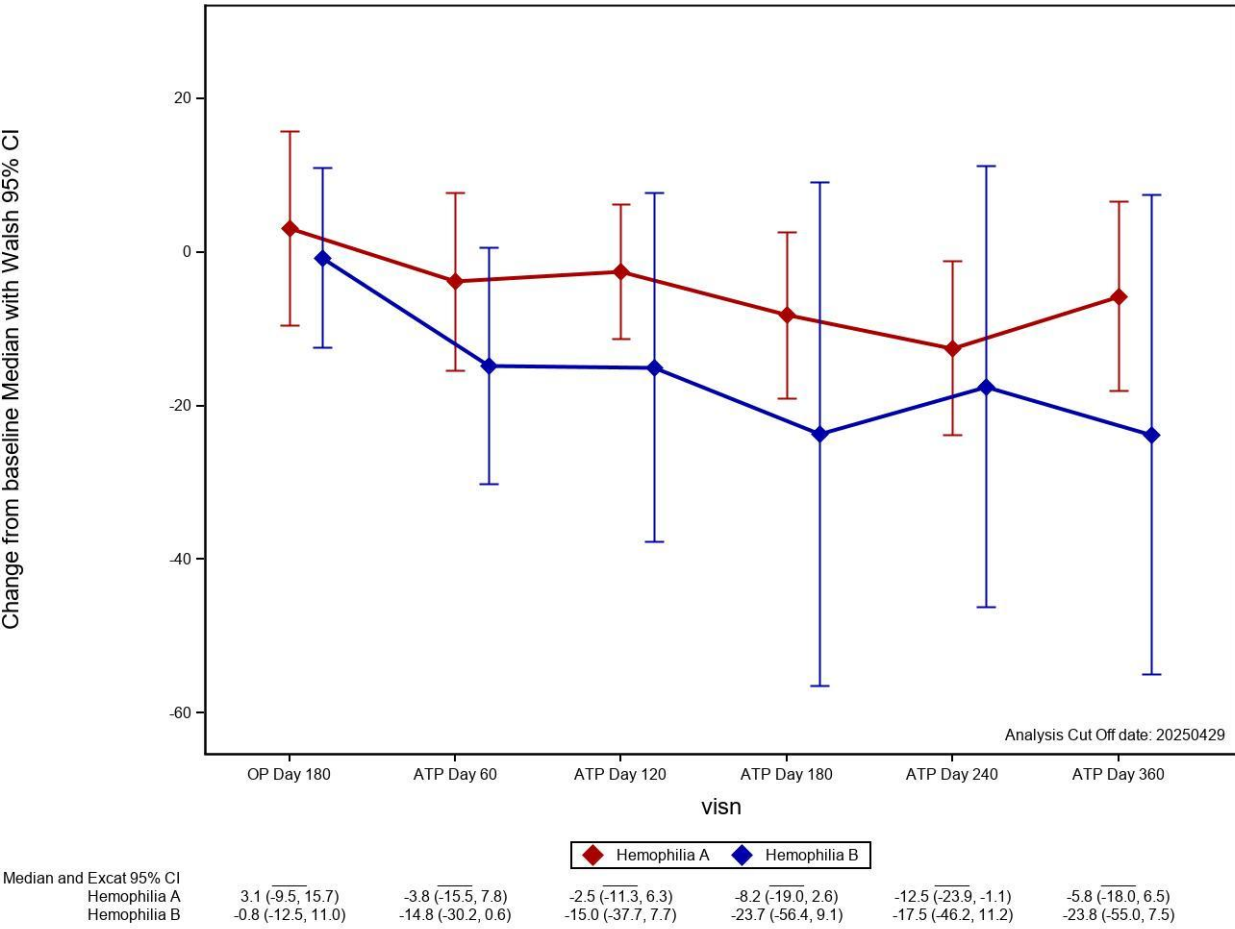
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.5.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Future - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



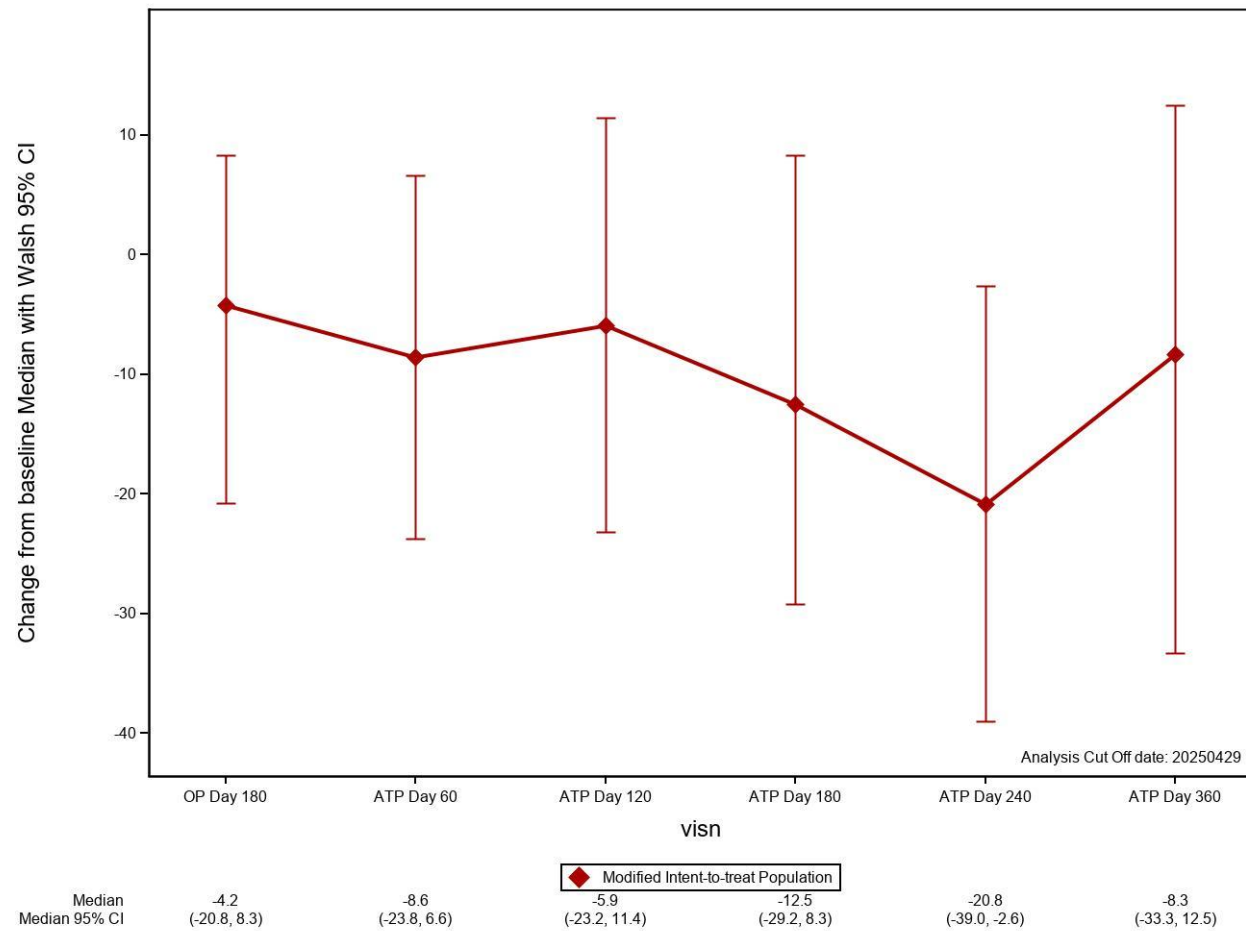
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.5.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Future - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



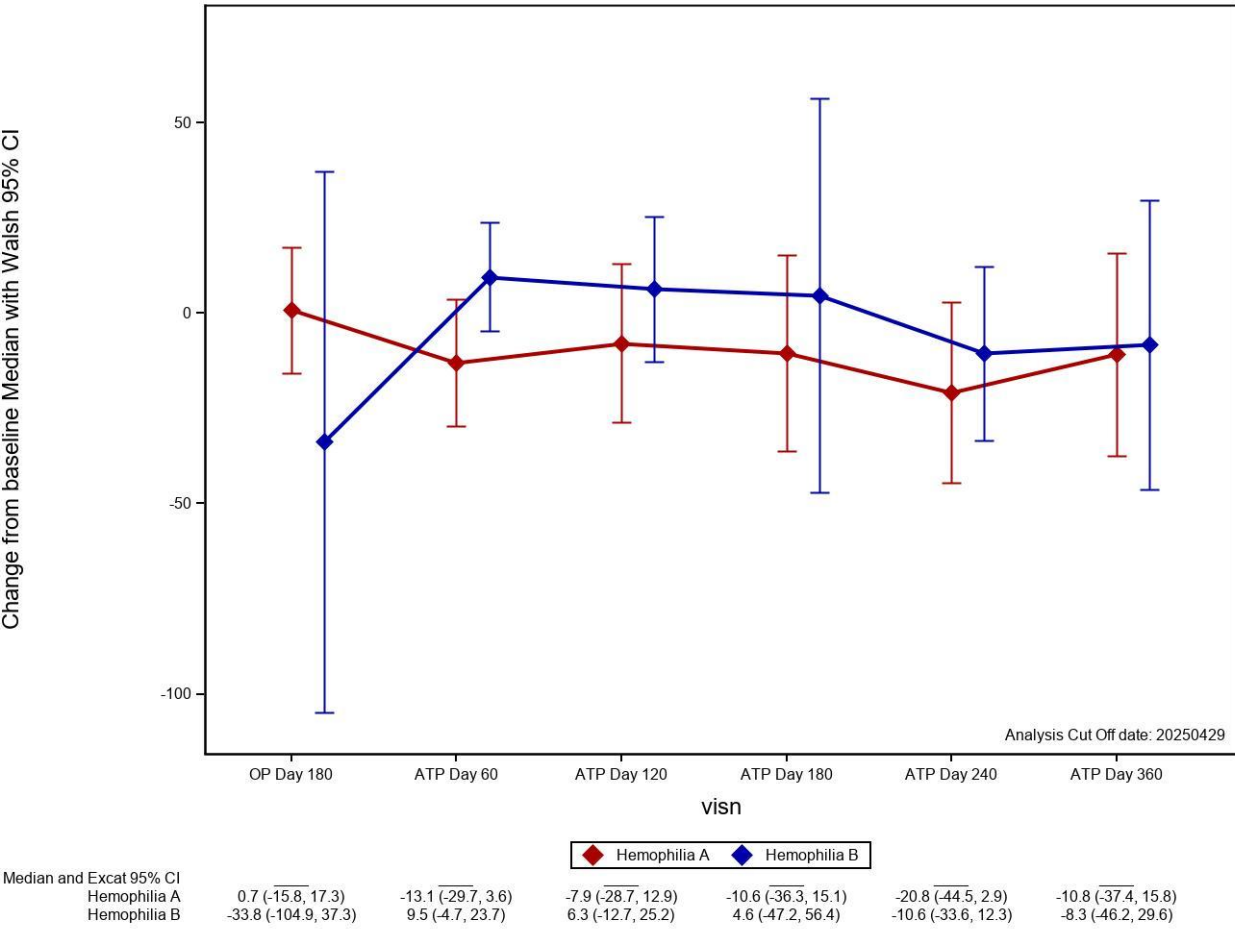
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.6.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Partnership and Sexuality - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



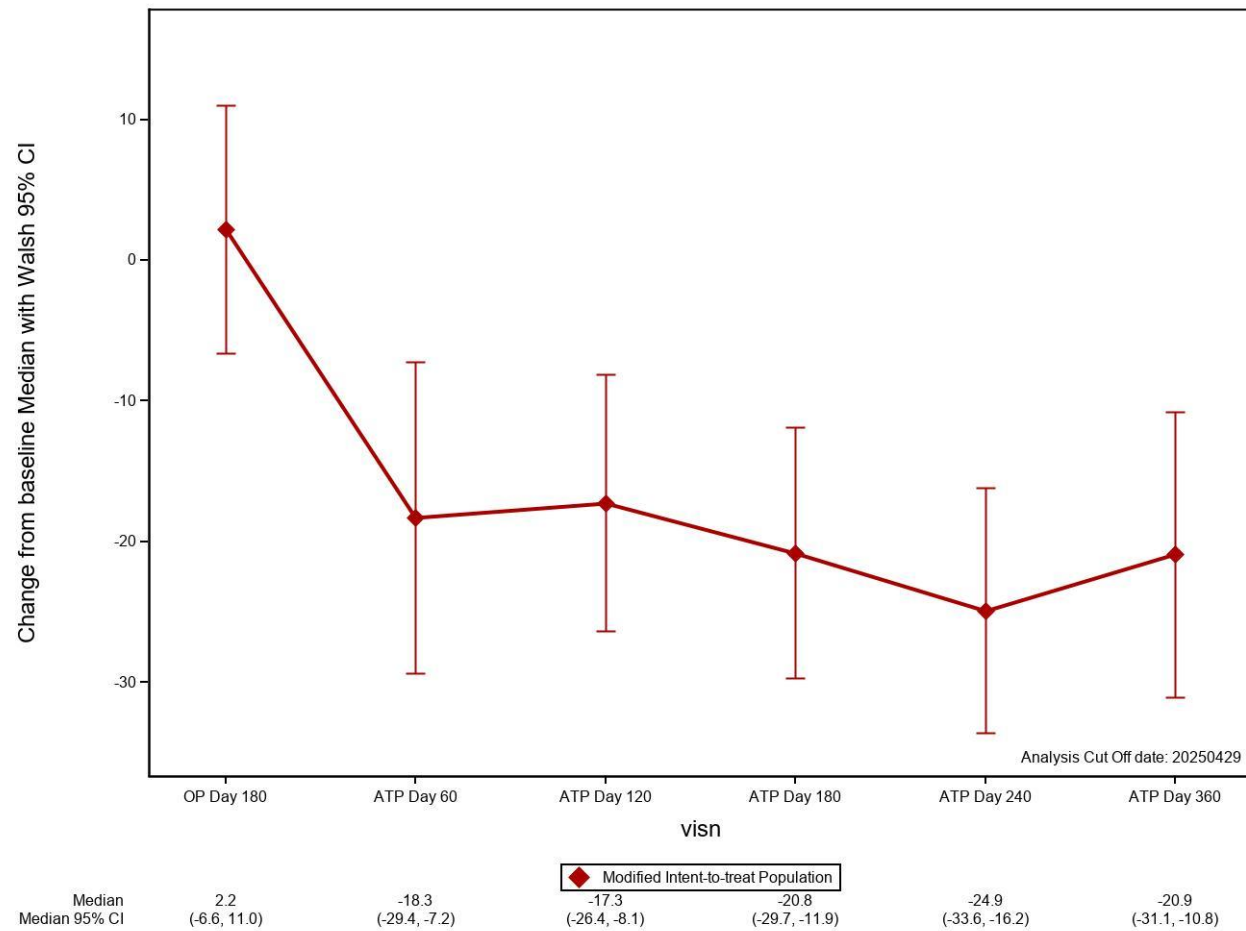
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.6.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Partnership and Sexuality - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



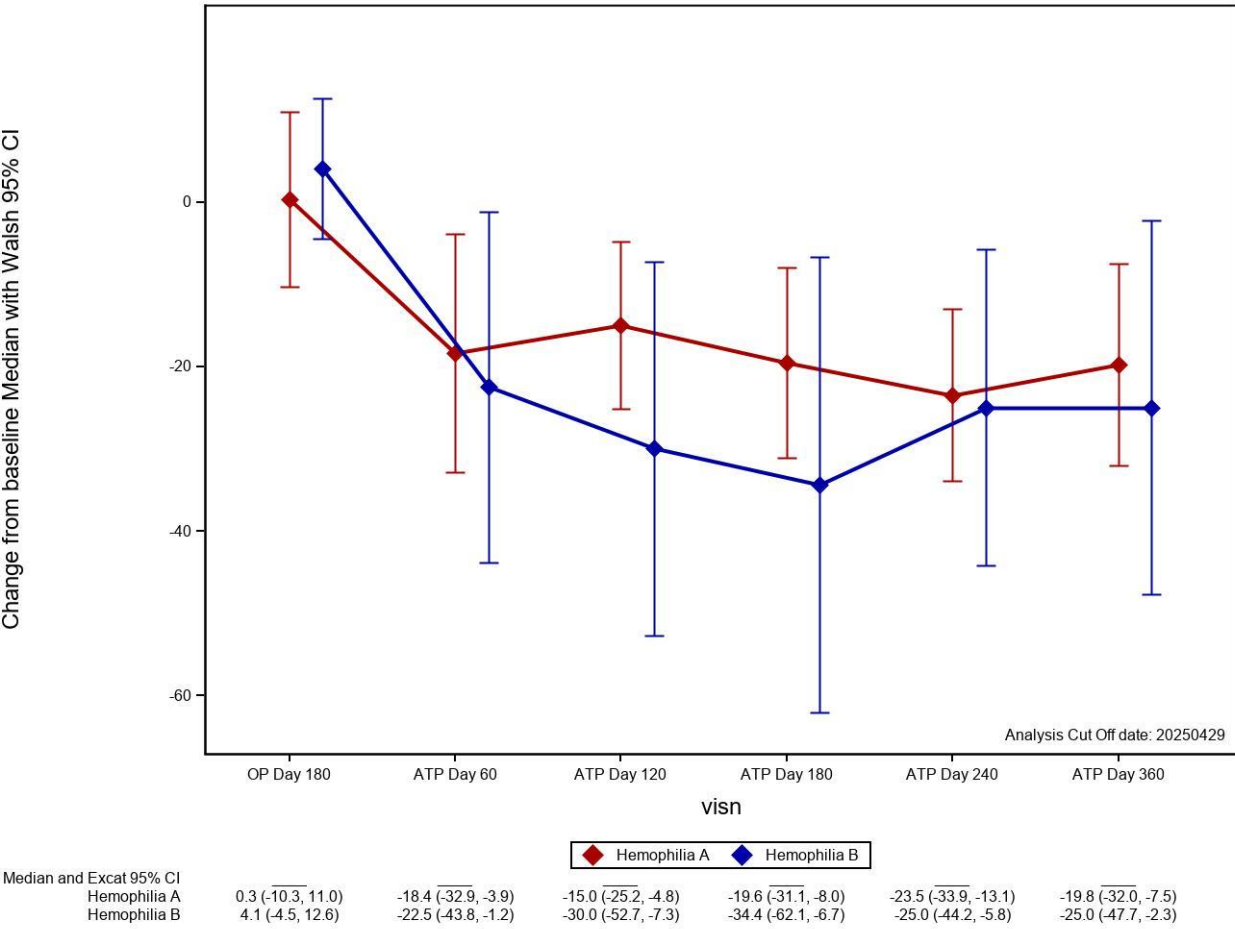
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.7.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Physical Health - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



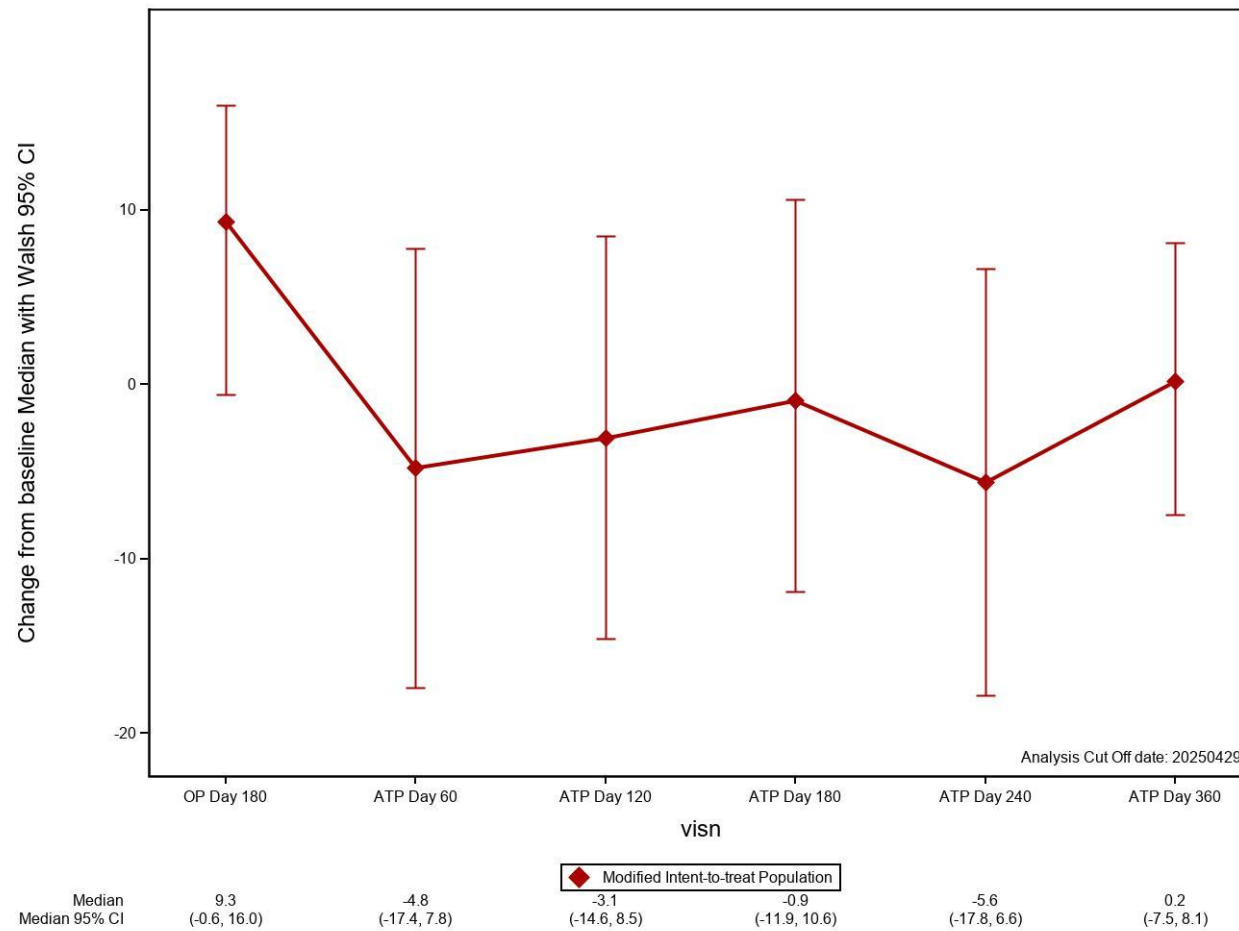
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.7.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Physical Health - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



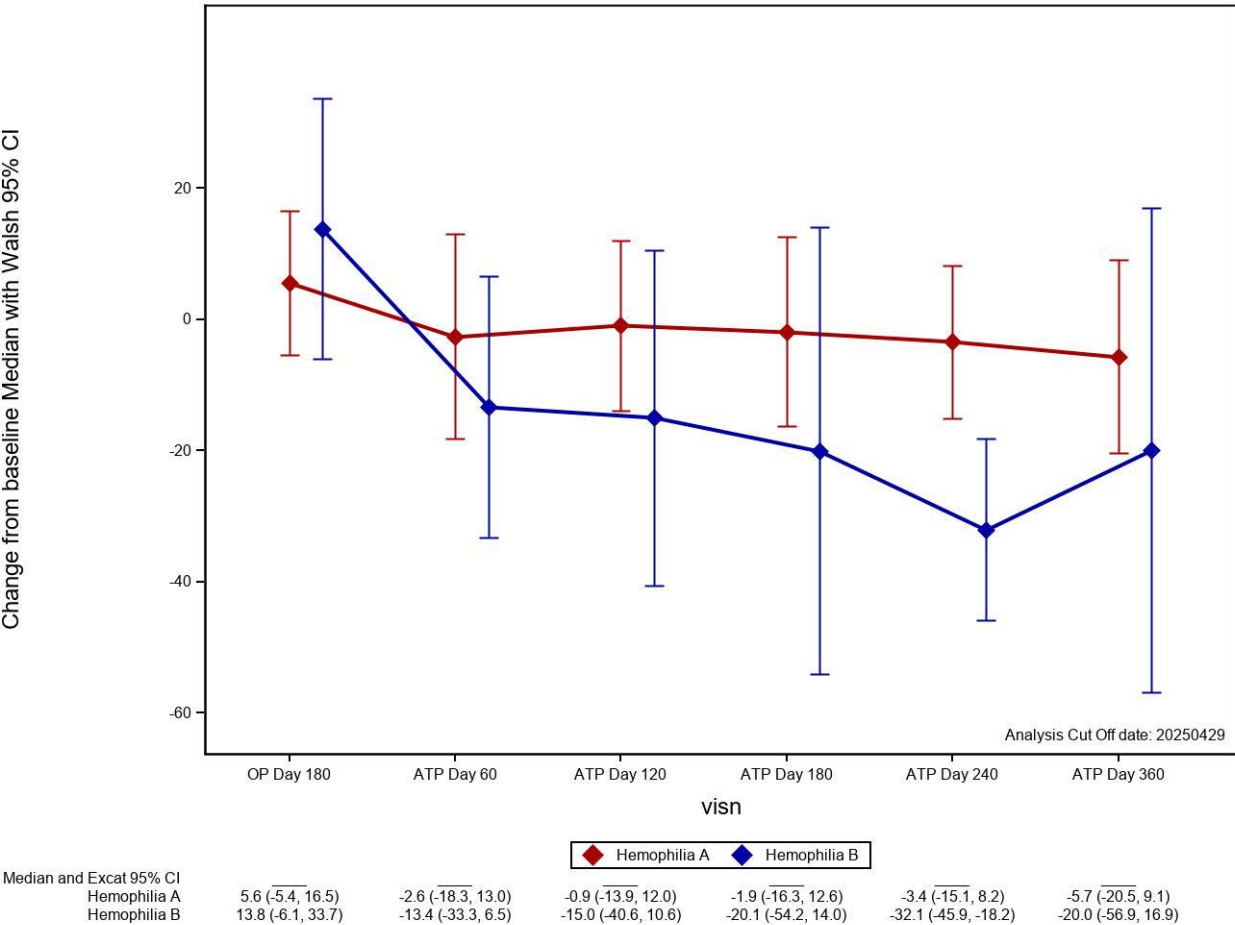
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.8.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Sport and Leisure – Modified Intent-to-Treat Set – Change from baseline Medians – Overall



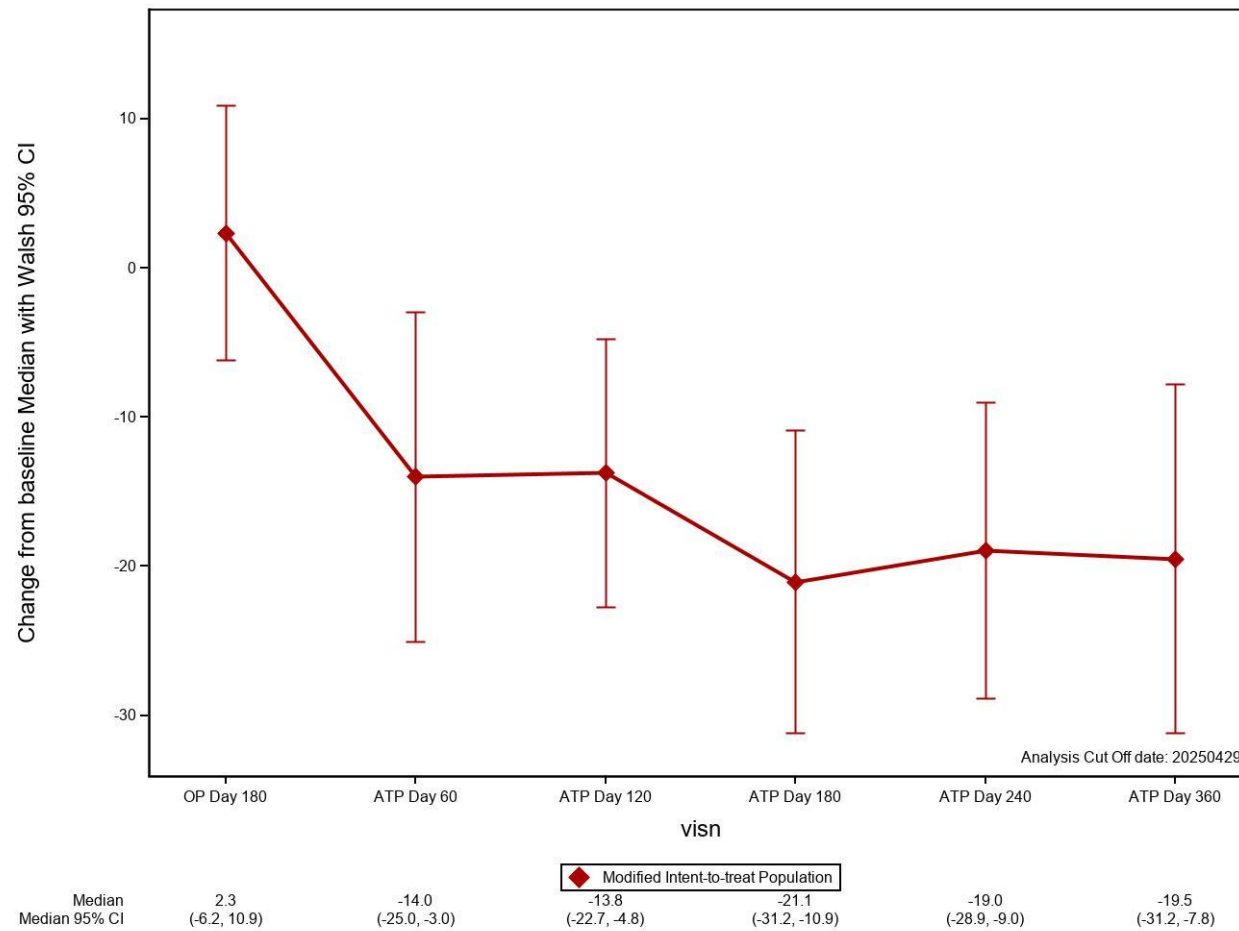
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.8.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Sport and Leisure – Modified Intent-to-Treat Set – Change from baseline Medians – By Disease Type



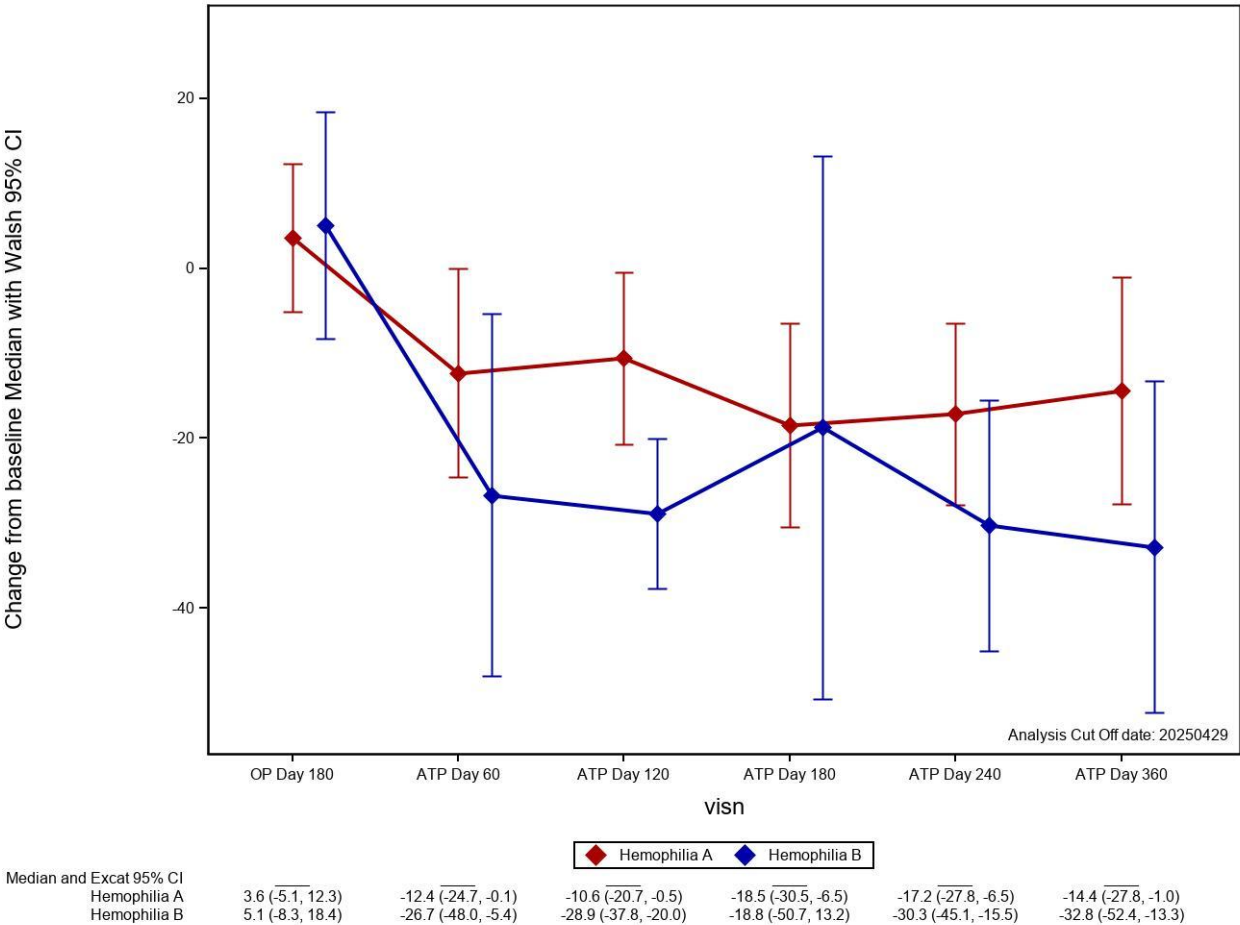
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.9.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) – Treatment – Modified Intent-to-Treat Set – Change from baseline Medians – Overall



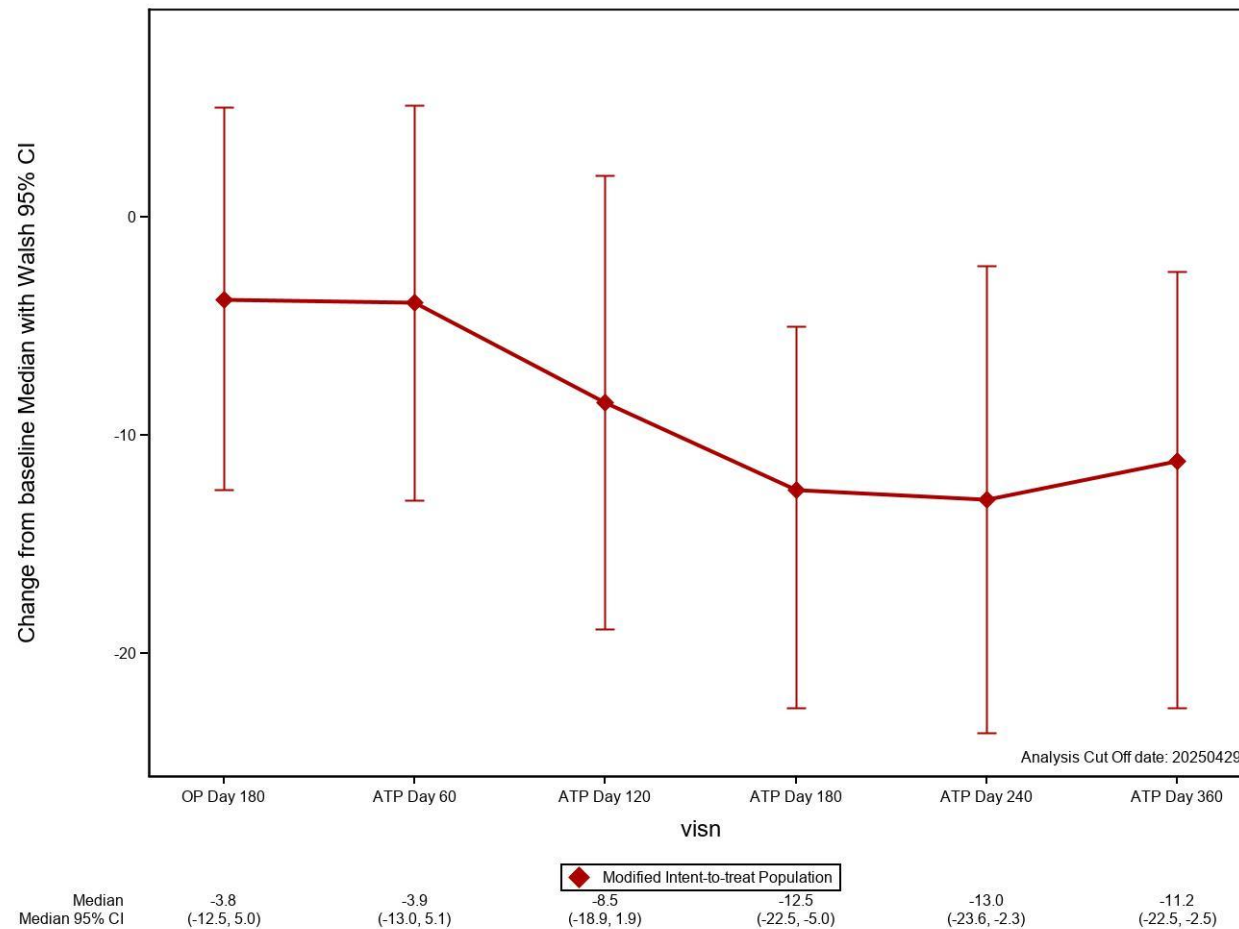
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.9.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Treatment - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



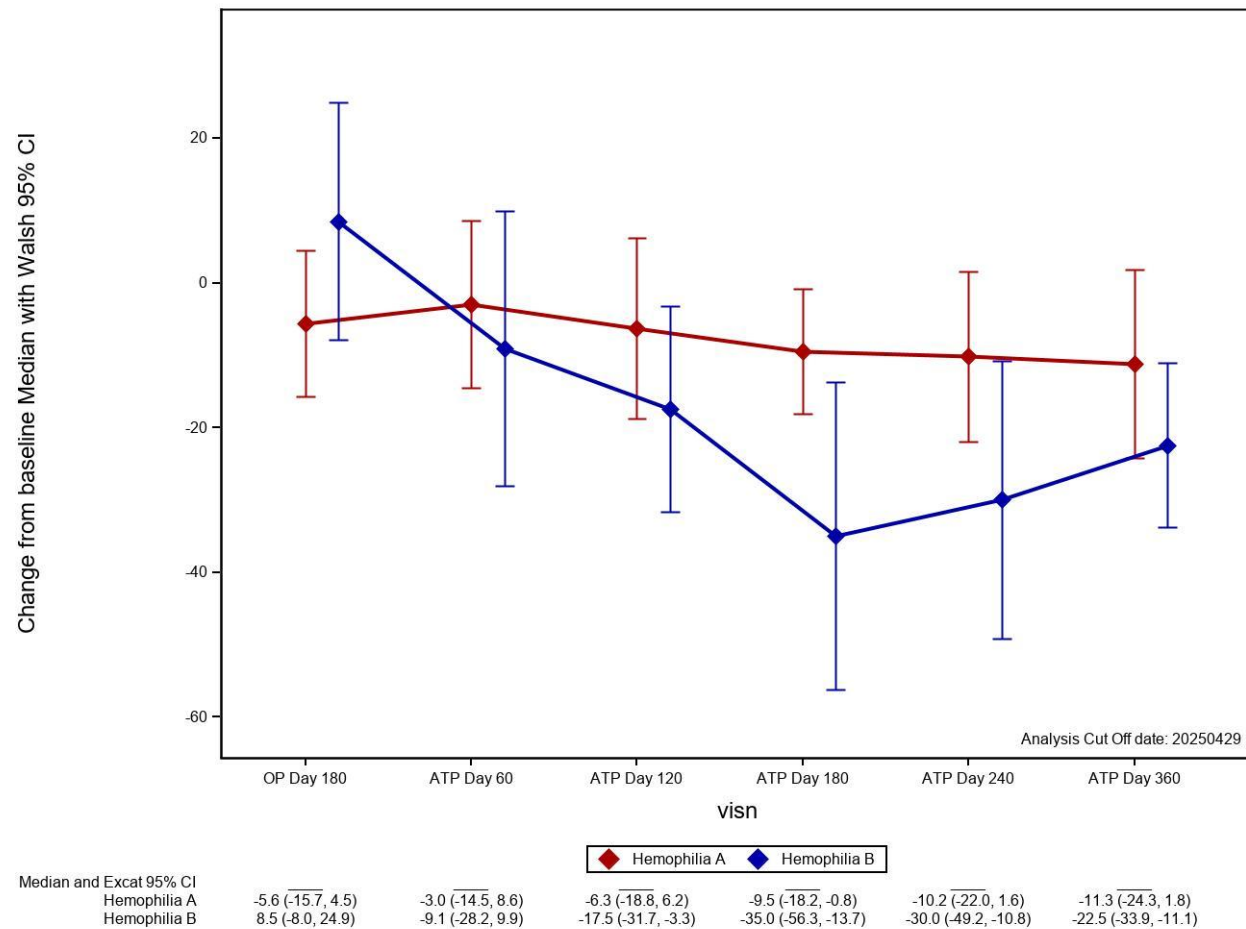
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.10.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - View of Yourself - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



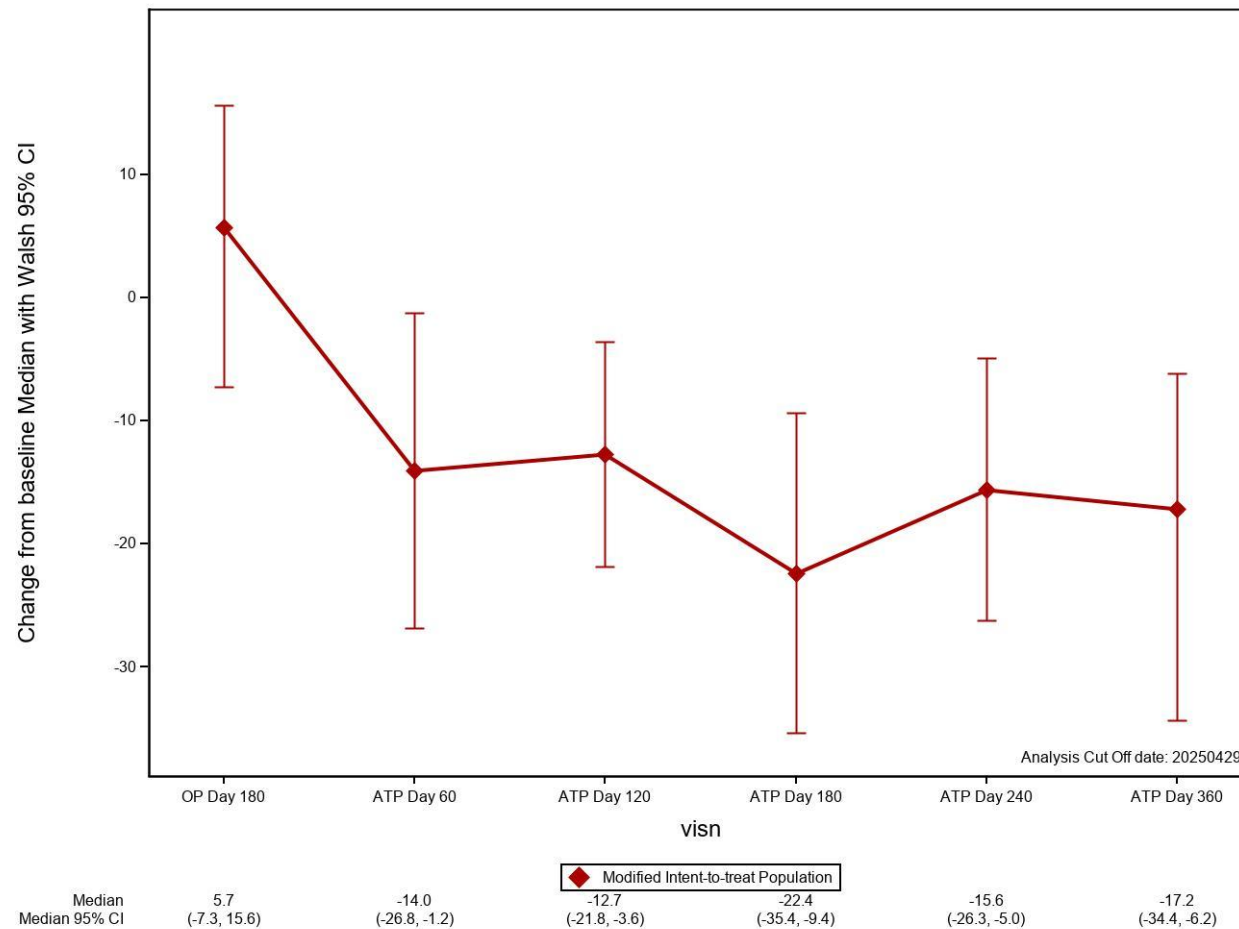
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.10.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - View of Yourself - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



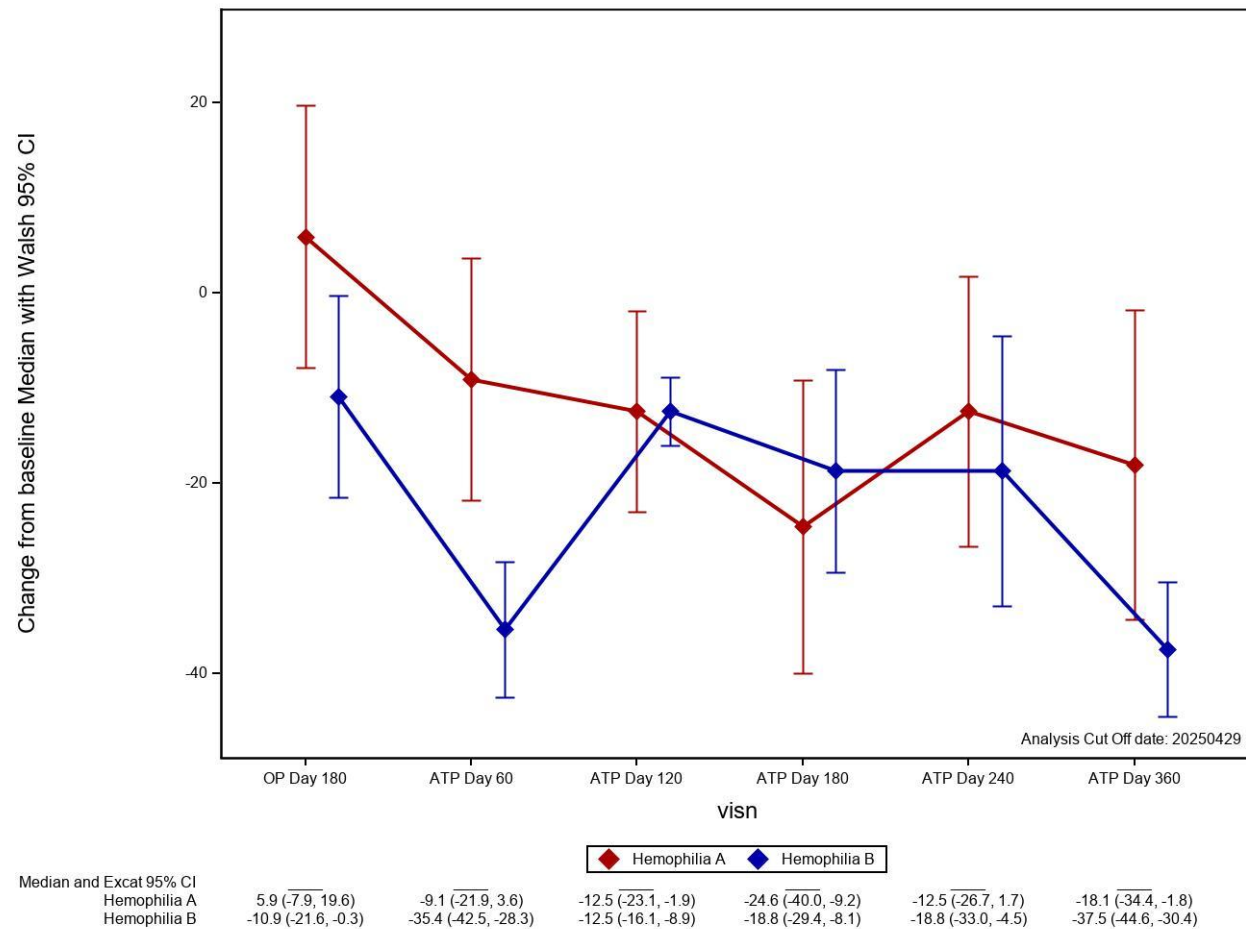
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.11.1: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Work and School - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



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

FIGURE .6.2.11.2: Hemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) - Work and School - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



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

8.2 Haem-QoL

8.2.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.1.3. Haem-QoL pediatric Total score response (≥ 15 points Improvement from baseline) analysis.

Modified Intent-to-Treat Set

Table page 1 of 1.

Event	N	Responders by phase			ATP 6-months vs. OP 6-months				OR Interacti on p- value [2]
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		
Overall									
-	13	1.0 (7.7%)	3.5 (26.9%)	3.2 (24.6%)	3.44 (0.63, 18.68) 0.1520	4.36 (0.66, 28.70) 0.1256	19.2 (-4.9, 43.4) 0.1180		
Disease type									
Hemophilia A	10	1.0 (10.0%)	3.2 (32.0%)	2.2 (22.0%)	3.18 (0.62, 16.37) 0.1669	4.21 (0.65, 27.34) 0.1317	22.0 (-5.0, 49.0) 0.1104		
Hemophilia B	3	0.0 (0.0%)	0.3 (10.0%)	1.0 (33.3%)	1.23 (0.08, 19.35) 0.8823	1.52 (0.02, 147.48) 0.8586	10.0 (-92.6, 112.6) 0.8483	0.6837	
Region									
Asia	6	0.0 (0.0%)	1.2 (20.0%)	1.2 (20.0%)	-	-	-		
Europe	3	1.0 (33.3%)	1.0 (33.3%)	0.0 (0.0%)	-	-	-		
Other	4	0.0 (0.0%)	1.3 (32.5%)	2.0 (50.0%)	-	-	-		-
White Race									
White	5	1.0 (20.0%)	1.3 (26.0%)	1.0 (20.0%)	-	-	-		
Other	8	0.0 (0.0%)	2.2 (27.5%)	2.2 (27.5%)	-	-	-		-

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visis data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD where performed by imputation, and the results were pooled using Rubin's rules.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 4.1.4. Haem-QoL pediatric Physical Health Domain score response (≥ 15 points Improvement from baseline) analysis.

Modified Intent-to-Treat Set

Table page 1 of 1.

Table page 2 of 2

		Responders by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)				
Overall								
-	13	4.3 (33.1%)	7.0 (53.8%)	7.7 (59.2%)	1.63 (0.59, 4.49) 0.3447	2.37 (0.41, 13.84) 0.3364	20.8 (-20.4, 61.9) 0.3224	
Disease type								
Hemophilia A	10	3.0 (30.0%)	6.2 (62.0%)	6.7 (67.0%)	2.06 (0.73, 5.86) 0.1743	3.82 (0.60, 24.27) 0.1549	32.0 (-8.9, 72.9) 0.1253	
Hemophilia B	3	1.3 (43.3%)	0.8 (26.7%)	1.0 (33.3%)	0.73 (0.05, 10.26) 0.8137	0.63 (0.01, 51.56) 0.8369	-11.1 (-109.9, 87.7) 0.8253	0.4590
Region								
Asia	6	2.0 (33.3%)	2.2 (36.7%)	2.7 (45.0%)	-	-	-	
Europe	3	2.0 (66.7%)	2.0 (66.7%)	2.0 (66.7%)	-	-	-	
Other	4	0.3 (7.5%)	2.8 (70.0%)	3.0 (75.0%)	-	-	-	-
White Race								
White	5	2.0 (40.0%)	3.8 (76.0%)	4.0 (80.0%)	-	-	-	
Other	8	2.3 (28.8%)	3.2 (40.0%)	3.7 (46.3%)	-	-	-	-

Results are based on multiple imputation for missing data. Parameter scores were all imputed (N=10 imputations) based on all visis data. The (observed or imputed) values at the corresponding visits were converted to binary responder data based on the required threshold. Descriptive results are based on averaging across the imputations, hence leading to decimals in n responders. The RR, OR and RD were performed by imputation, and the results were pooled using Rubin's rules.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.2. Haem-QoL response (≥ 15 points Improvement from baseline) analysis.
 Modified Intent-to-Treat Set

Table page 1 of 11.

		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Attitude/View										
Overall										
Set Modified Intent-to-Treat	13	3 (23.1%)	3 (23.1%)	5 (38.5%)	1.00 (0.27, 3.69) 1.0000		1.00 (0.18, 5.47) 1.0000		-0.0 (-30.2, 30.2) 1.0000	
Disease type										
Hemophilia A	10	2 (20.0%)	3 (30.0%)	4 (40.0%)	1.50 (0.38, 6.00) 0.5664		1.71 (0.28, 10.59) 0.5618		10.0 (-23.4, 43.4) 0.5571	
Hemophilia B	3	1 (33.3%)	0 (0.0%)	1 (33.3%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	
Region										
Asia	6	1 (16.7%)	1 (16.7%)	3 (50.0%)	-		-		-	
Europe	3	1 (33.3%)	2 (66.7%)	0 (0.0%)	-		-		-	
Other	4	1 (25.0%)	0 (0.0%)	2 (50.0%)	-		-		-	
White Race										
White	5	2 (40.0%)	2 (40.0%)	1 (20.0%)	-		-		-	
Other	8	1 (12.5%)	1 (12.5%)	4 (50.0%)	-		-		-	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 4.2.2. Haem-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 2 of 11.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
DOMAIN: Coping/Dealing								
Overall								
Modified Intent-to-Treat Set	13	1 (7.7%)	4 (30.8%)	3 (23.1%)	4.00 (0.45, 35.79) 0.2150	5.33 (0.42, 67.92) 0.1972	23.1 (-8.2, 54.4) 0.1483	
Disease type								
Hemophilia A	10	1 (10.0%)	4 (40.0%)	3 (30.0%)	4.00 (0.45, 35.79) 0.2150	6.00 (0.40, 89.31) 0.1934	30.0 (-9.7, 69.7) 0.1384	
Region								
Asia	6	0 (0.0%)	2 (33.3%)	2 (33.3%)	-	-	-	
Europe	3	1 (33.3%)	0 (0.0%)	0 (0.0%)	-	-	-	
Other	4	0 (0.0%)	2 (50.0%)	1 (25.0%)	-	-	-	-
White Race								
White	5	1 (20.0%)	1 (20.0%)	1 (20.0%)	-	-	-	
Other	8	0 (0.0%)	3 (37.5%)	2 (25.0%)	-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.2. Haem-QoL response (≥ 15 points Improvement from baseline) analysis
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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Family										
Overall										
Modified Intent-to-Treat Set	13	3 (23.1%)	4 (30.8%)	4 (30.8%)	1.33 (0.30, 5.96) 0.7064		1.48 (0.19, 11.39) 0.7057		7.7 (-32.0, 47.4) 0.7039	
Disease type										
Hemophilia A	10	3 (30.0%)	3 (30.0%)	3 (30.0%)	1.00 (0.20, 4.95) 1.0000		1.00 (0.10, 9.84) 1.0000		0.0 (-48.0, 48.0) 1.0000	
Hemophilia B	3	0 (0.0%)	1 (33.3%)	1 (33.3%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	
Region										
Asia	6	2 (33.3%)	1 (16.7%)	1 (16.7%)	-		-		-	
Europe	3	0 (0.0%)	1 (33.3%)	1 (33.3%)	-		-		-	
Other	4	1 (25.0%)	2 (50.0%)	2 (50.0%)	-		-		-	
White Race										
White	5	1 (20.0%)	1 (20.0%)	1 (20.0%)	-		-		-	
Other	8	2 (25.0%)	3 (37.5%)	3 (37.5%)	-		-		-	

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Feelings									
Overall									
Modified Intent-to-Treat Set	13	3 (23.1%)	4 (30.8%)	5 (38.5%)	1.33 (0.38, 4.72) 0.6558		1.48 (0.27, 8.28) 0.6543	7.7 (-25.8, 41.1) 0.6522	
Disease type									
Hemophilia A	10	2 (20.0%)	4 (40.0%)	3 (30.0%)	2.00 (0.50, 8.00) 0.3270		2.67 (0.40, 17.79) 0.3111	20.0 (-17.2, 57.2) 0.2918	
Hemophilia B	3	1 (33.3%)	0 (0.0%)	2 (66.7%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000	0.0 (-92.4, 92.4) 1.0000	0.6838
Region									
Asia	6	1 (16.7%)	1 (16.7%)	1 (16.7%)	-		-	-	
Europe	3	2 (66.7%)	2 (66.7%)	1 (33.3%)	-		-	-	
Other	4	0 (0.0%)	1 (25.0%)	3 (75.0%)	-		-	-	-
White Race									
White	5	2 (40.0%)	2 (40.0%)	2 (40.0%)	-		-	-	
Other	8	1 (12.5%)	2 (25.0%)	3 (37.5%)	-		-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		
DOMAIN: Friends									
Overall									
Modified Intent-to-Treat Set	13	1 (7.7%)	4 (30.8%)	3 (23.1%)	4.00 (0.45, 35.79) 0.2150	5.33 (0.42, 67.92) 0.1972	23.1 (-8.2, 54.4) 0.1483		
Disease type									
Hemophilia A	10	0 (0.0%)	4 (40.0%)	3 (30.0%)	5.00 (0.87, 28.86) 0.0720	9.00 (1.19, 68.13) 0.0334	40.0 (9.6, 70.4) 0.0098		
Hemophilia B	3	1 (33.3%)	0 (0.0%)	0 (0.0%)	1.00 (0.06, 15.99) 1.0000	1.00 (0.02, 63.93) 1.0000	0.0 (-92.4, 92.4) 1.0000		0.4234
Region									
Asia	6	0 (0.0%)	2 (33.3%)	2 (33.3%)	-	-	-		
Europe	3	1 (33.3%)	0 (0.0%)	0 (0.0%)	-	-	-		
Other	4	0 (0.0%)	2 (50.0%)	1 (25.0%)	-	-	-		-
White Race									
White	5	1 (20.0%)	1 (20.0%)	1 (20.0%)	-	-	-		
Other	8	0 (0.0%)	3 (37.5%)	2 (25.0%)	-	-	-		-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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Event	N	Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]
DOMAIN: Future								
Overall								
Modified Intent-to-Treat Set	13	2 (15.4%)	1 (7.7%)	1 (7.7%)	0.50 (0.05, 5.51) 0.5714	0.46 (0.03, 6.69) 0.5685	-7.7 (-33.5, 18.1) 0.5586	
Disease type								
Hemophilia A	10	1 (10.0%)	1 (10.0%)	1 (10.0%)	1.00 (0.06, 15.99) 1.0000	1.00 (0.05, 21.75) 1.0000	-0.0 (-27.7, 27.7) 1.0000	
Hemophilia B	3	1 (33.3%)	0 (0.0%)	0 (0.0%)	1.00 (0.06, 15.99) 1.0000	1.00 (0.02, 63.93) 1.0000	0.0 (-92.4, 92.4) 1.0000	1.0000
Region								
Asia	6	0 (0.0%)	1 (16.7%)	0 (0.0%)	-	-	-	
Europe	3	2 (66.7%)	0 (0.0%)	0 (0.0%)	-	-	-	
Other	4	0 (0.0%)	0 (0.0%)	1 (25.0%)	-	-	-	-
White Race								
White	5	2 (40.0%)	0 (0.0%)	0 (0.0%)	-	-	-	
Other	8	0 (0.0%)	1 (12.5%)	1 (12.5%)	-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months					OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		
DOMAIN: Other People									
Overall									
Modified Intent-to-Treat Set	13	1 (7.7%)	3 (23.1%)	4 (30.8%)	3.00 (0.31, 28.84) 0.3414	3.60 (0.27, 47.53) 0.3306	15.4 (-13.6, 44.4) 0.2980		
Disease type									
Hemophilia A	10	1 (10.0%)	3 (30.0%)	3 (30.0%)	3.00 (0.31, 28.84) 0.3414	3.86 (0.26, 57.68) 0.3280	20.0 (-17.2, 57.2) 0.2918		
Hemophilia B	3	0 (0.0%)	0 (0.0%)	1 (33.3%)	1.00 (0.06, 15.99) 1.0000	1.00 (0.02, 63.93) 1.0000	0.0 (-92.4, 92.4) 1.0000		0.6059
Region									
Asia	6	0 (0.0%)	1 (16.7%)	2 (33.3%)	-	-	-		
Europe	3	1 (33.3%)	1 (33.3%)	0 (0.0%)	-	-	-		
Other	4	0 (0.0%)	1 (25.0%)	2 (50.0%)	-	-	-		-
White Race									
White	5	1 (20.0%)	1 (20.0%)	1 (20.0%)	-	-	-		
Other	8	0 (0.0%)	2 (25.0%)	3 (37.5%)	-	-	-		-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Perceived Support										
Overall										
Set										
Modified Intent-to-Treat	13	3 (23.1%)	2 (15.4%)	4 (30.8%)	0.67 (0.17, 2.67) 0.5664		0.61 (0.11, 3.30) 0.5627		-7.7 (-33.5, 18.1) 0.5586	
Disease type										
Hemophilia A	10	2 (20.0%)	2 (20.0%)	4 (40.0%)	1.00 (0.25, 4.00) 1.0000		1.00 (0.18, 5.65) 1.0000		-0.0 (-27.7, 27.7) 1.0000	
Hemophilia B	3	1 (33.3%)	0 (0.0%)	0 (0.0%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	1.0000
Region										
Asia	6	1 (16.7%)	1 (16.7%)	4 (66.7%)	-		-		-	
Europe	3	2 (66.7%)	0 (0.0%)	0 (0.0%)	-		-		-	
Other	4	0 (0.0%)	1 (25.0%)	0 (0.0%)	-		-		-	-
White Race										
White	5	2 (40.0%)	0 (0.0%)	0 (0.0%)	-		-		-	
Other	8	1 (12.5%)	2 (25.0%)	4 (50.0%)	-		-		-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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		Incidences by phase			ATP 6-months vs. OP 6-months						
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]	
Event	N	n (%)	n (%)	n (%)							
DOMAIN: Relationship											
Overall											
Modified Intent-to-Treat Set	13	5 (38.5%)	2 (15.4%)	2 (15.4%)	0.40 (0.08, 2.06) 0.2734		0.29 (0.03, 2.52) 0.2619		-23.1 (-60.9, 14.8) 0.2323		
Disease type											
Hemophilia A	10	3 (30.0%)	2 (20.0%)	2 (20.0%)	0.67 (0.11, 3.99) 0.6569		0.58 (0.05, 6.22) 0.6554		-10.0 (-53.4, 33.4) 0.6514		
Hemophilia B	3	2 (66.7%)	0 (0.0%)	0 (0.0%)	0.50 (0.05, 5.51) 0.5714		0.25 (0.00, 30.41) 0.5714		-33.3 (-140.0, 73.4) 0.5403		0.5902
Region											
Asia	6	2 (33.3%)	1 (16.7%)	1 (16.7%)	-		-		-		
Europe	3	2 (66.7%)	1 (33.3%)	1 (33.3%)	-		-		-		
Other	4	1 (25.0%)	0 (0.0%)	0 (0.0%)	-		-		-		-
White Race											
White	5	3 (60.0%)	1 (20.0%)	1 (20.0%)	-		-		-		
Other	8	2 (25.0%)	1 (12.5%)	1 (12.5%)	-		-		-		-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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		Incidences by phase			ATP 6-months vs. OP 6-months					
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]		RD (95%CI) p-value [3]	OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)						
DOMAIN: Sports and School										
Overall										
Set Modified Intent-to-Treat	13	3 (23.1%)	2 (15.4%)	3 (23.1%)	0.67 (0.30, 1.48) 0.3206		0.61 (0.23, 1.57) 0.3030		-7.7 (-22.2, 6.8) 0.2980	
Disease type										
Hemophilia A	10	3 (30.0%)	2 (20.0%)	2 (20.0%)	0.67 (0.30, 1.48) 0.3206		0.58 (0.21, 1.61) 0.2977		-10.0 (-28.6, 8.6) 0.2918	
Hemophilia B	3	0 (0.0%)	0 (0.0%)	1 (33.3%)	1.00 (0.06, 15.99) 1.0000		1.00 (0.02, 63.93) 1.0000		0.0 (-92.4, 92.4) 1.0000	0.8079
Region										
Asia	6	1 (16.7%)	1 (16.7%)	1 (16.7%)	-		-		-	
Europe	3	1 (33.3%)	1 (33.3%)	1 (33.3%)	-		-		-	
Other	4	1 (25.0%)	0 (0.0%)	1 (25.0%)	-		-		-	-
White Race										
White	5	2 (40.0%)	1 (20.0%)	2 (40.0%)	-		-		-	
Other	8	1 (12.5%)	1 (12.5%)	1 (12.5%)	-		-		-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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 TABLE 4.2.2. Haem-QoL response (≥ 15 points Improvement from baseline) analysis
 Modified Intent-to-Treat Set

Table page 11 of 11.

		Incidences by phase			ATP 6-months vs. OP 6-months			
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months				OR Interacti on p- value [2]
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	
DOMAIN: Treatment								
Overall								
Modified Intent-to-Treat Set	13	2 (15.4%)	5 (38.5%)	5 (38.5%)	2.50 (0.63, 10.00) 0.1950	3.44 (0.58, 20.44) 0.1746	23.1 (-8.2, 54.4) 0.1483	
Disease type								
Hemophilia A	10	2 (20.0%)	4 (40.0%)	4 (40.0%)	2.00 (0.50, 8.00) 0.3270	2.67 (0.40, 17.79) 0.3111	20.0 (-17.2, 57.2) 0.2918	
Hemophilia B	3	0 (0.0%)	1 (33.3%)	1 (33.3%)	1.00 (0.06, 15.99) 1.0000	1.00 (0.02, 63.93) 1.0000	0.0 (-92.4, 92.4) 1.0000	0.6838
Region								
Asia	6	2 (33.3%)	2 (33.3%)	3 (50.0%)	-	-	-	
Europe	3	0 (0.0%)	1 (33.3%)	0 (0.0%)	-	-	-	
Other	4	0 (0.0%)	2 (50.0%)	2 (50.0%)	-	-	-	-
White Race								
White	5	0 (0.0%)	1 (20.0%)	1 (20.0%)	-	-	-	
Other	8	2 (25.0%)	4 (50.0%)	4 (50.0%)	-	-	-	-

Missing scores were imputed using LOCF. In case no assessments were done for all of the visits, the subject was not used in the analysis.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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8.2.2 Sensitivitätsanalyse

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 TABLE 6.1.12. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Total Score.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 42.7 (14.81) 31.5 46.75 50.3	-	-
OP Day 180	n=12 42.7 (12.81) 33.3 43.34 53.7	n=12 -0.1 (11.74) -9.4 -2.60 9.1	-0.1 (-9.1, 9.0)
ATP Baseline	n=13 41.6 (12.67) 29.9 41.56 53.2	-	-
ATP Day 180	n=11 34.0 (11.60) 23.4 28.25 49.4	n=11 -8.6 (7.11) -15.3 -6.17 -3.9	-7.9 (-13.7, -2.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-8.3 (-18.2, 1.6) 0.1000
ATP Day 360	n=12 32.0 (13.40) 21.9 29.06 45.6	n=12 -10.7 (7.75) -15.7 -10.71 -5.0	-10.3 (-15.9, -4.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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Pfizer Inc.: Final
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 TABLE 6.1.12. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Total Score
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 44.8 (16.23) 31.5 48.54 52.3	-	-
OP Day 180	n=10 43.6 (12.25) 40.3 43.34 55.5	n=10 -1.2 (10.27) -8.4 -2.60 8.8	-1.1 (-12.5, 10.2)
ATP Baseline	n=10 44.1 (12.50) 29.9 45.78 54.5	-	-
ATP Day 180	n=9 35.9 (12.06) 25.6 34.74 49.4	n=9 -9.8 (6.78) -15.3 -6.17 -4.9	-8.7 (-15.2, -2.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-7.4 (-17.0, 2.1)
ATP Day 360	n=9 34.7 (14.43) 25.0 34.42 47.7	n=9 -10.9 (5.79) -14.9 -11.69 -5.8	0.1120 -10.8 (-17.0, -4.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.12. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Total Score
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 35.4 (5.32) 30.8 34.09 41.2	-	-
OP Day 180	n=2 37.8 (19.97) 23.7 37.82 51.9	n=2 5.4 (22.27) -10.4 5.36 21.1	1.0 (-16.9, 18.9)
ATP Baseline	n=3 33.5 (11.53) 20.8 36.69 43.2	-	-
ATP Day 180	n=2 25.6 (3.21) 23.4 25.65 27.9	n=2 -3.1 (8.04) -8.8 -3.08 2.6	-8.1 (-19.6, 3.3)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-9.1 (-38.4, 20.3)
ATP Day 360	n=3 23.6 (4.01) 19.2 24.68 26.9	n=3 -10.0 (13.96) -24.0 -9.74 3.9	0.5059 -9.9 (-25.8, 6.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.13. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Attitude/View.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 47.1 (22.59) 32.5 50.00 62.5	-	-
OP Day 180	n=12 49.6 (20.61) 28.8 56.25 67.5	n=12 2.3 (23.15) -15.0 1.25 16.3	0.6 (-15.0, 20.0)
ATP Baseline	n=13 46.2 (14.85) 37.5 50.00 57.5	-	-
ATP Day 180	n=11 34.8 (14.07) 17.5 40.00 45.0	n=11 -12.7 (7.28) -20.0 -10.00 -10.0	-12.5 (-18.8, -2.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-11.9 (-25.0, 2.5)
ATP Day 360	n=12 32.3 (18.32) 18.8 28.75 48.8	n=12 -15.4 (12.92) -28.8 -12.50 -3.8	0.0659 -16.9 (-27.5, -5.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.13. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Attitude/View
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 51.3 (23.25) 32.5 56.25 65.0	-	-
OP Day 180	n=10 51.3 (19.37) 30.0 56.25 67.5	n=10 0.0 (18.10) -12.5 1.25 12.5	0.0 (-17.8, 17.8)
ATP Baseline	n=10 50.8 (11.31) 45.0 53.75 57.5	-	-
ATP Day 180	n=9 38.9 (11.93) 35.0 40.00 45.0	n=9 -14.4 (6.47) -20.0 -10.00 -10.0	-12.9 (-18.0, -7.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-12.5 (-26.0, 1.0)
ATP Day 360	n=9 36.7 (19.36) 22.5 42.50 50.0	n=9 -16.7 (12.37) -27.5 -12.50 -5.0	0.0665 -17.5 (-28.7, -6.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.13. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Attitude/View
 Modified Intent-to-Treat Set

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Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 33.3 (16.07) 15.0 40.00 45.0	-	-
OP Day 180	n=2 41.3 (33.59) 17.5 41.25 65.0	n=2 13.8 (51.27) -22.5 13.75 50.0	5.0 (-36.2, 46.2)
ATP Baseline	n=3 30.8 (17.02) 17.5 25.00 50.0	-	-
ATP Day 180	n=2 16.3 (1.77) 15.0 16.25 17.5	n=2 -5.0 (7.07) -10.0 -5.00 0.0	-21.9 (-35.4, -8.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-18.4 (-78.8, 42.0)
ATP Day 360	n=3 19.2 (1.44) 17.5 20.00 20.0	n=3 -11.7 (16.65) -30.0 -7.50 2.5	0.5119 -10.6 (-29.1, 7.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.14. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Coping/Dealing.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 31.3 (19.30) 14.3 32.14 46.4	-	-
OP Day 180	n=12 32.7 (21.26) 17.9 28.57 48.2	n=12 1.8 (13.83) -8.9 1.79 14.3	1.8 (-7.1, 10.7)
ATP Baseline	n=13 35.2 (21.32) 21.4 35.71 50.0	-	-
ATP Day 180	n=11 33.1 (22.59) 17.9 28.57 46.4	n=11 -4.5 (18.63) -25.0 0.00 7.1	-7.1 (-23.2, 7.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-8.9 (-26.8, 10.7) 0.3003
ATP Day 360	n=12 31.0 (21.72) 12.5 28.57 46.4	n=12 -4.8 (14.31) -14.3 -1.79 1.8	-7.1 (-21.4, 8.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.14. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Coping/Dealing
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 34.6 (20.07) 14.3 33.93 53.6	-	-
OP Day 180	n=10 38.2 (18.75) 21.4 37.50 50.0	n=10 3.6 (14.48) -7.1 7.14 14.3	3.6 (-8.6, 15.8)
ATP Baseline	n=10 40.7 (19.65) 25.0 41.07 53.6	-	-
ATP Day 180	n=9 37.7 (21.66) 28.6 35.71 46.4	n=9 -4.4 (20.65) -25.0 0.00 7.1	-7.1 (-30.0, 15.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-12.6 (-38.2, 12.9)
ATP Day 360	n=9 35.3 (23.24) 21.4 39.29 46.4	n=9 -6.7 (15.61) -17.9 -3.57 0.0	0.2963 -11.3 (-28.0, 5.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.14. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Coping/Dealing
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 20.2 (13.52) 10.7 14.29 35.7	-	-
OP Day 180	n=2 5.4 (2.53) 3.6 5.36 7.1	n=2 -7.1 (5.05) -10.7 -7.14 -3.6	-13.8 (-29.1, 1.4)
ATP Baseline	n=3 16.7 (17.98) 0.0 14.29 35.7	-	-
ATP Day 180	n=2 12.5 (17.68) 0.0 12.50 25.0	n=2 -5.4 (7.58) -10.7 -5.36 0.0	-6.3 (-11.3, -1.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	10.7 (0.6, 20.9) 0.0405
ATP Day 360	n=3 17.9 (9.45) 10.7 14.29 28.6	n=3 1.2 (8.99) -7.1 0.00 10.7	1.8 (-8.4, 11.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.15. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Family.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 43.0 (26.77) 21.9 50.00 53.1	-	-
OP Day 180	n=12 43.2 (20.98) 25.0 42.19 51.6	n=12 -1.0 (18.24) -18.8 3.13 9.4	-0.8 (-18.8, 14.1)
ATP Baseline	n=13 39.9 (22.10) 25.0 37.50 50.0	-	-
ATP Day 180	n=11 36.1 (19.83) 25.0 37.50 50.0	n=11 -7.7 (23.73) -25.0 -6.25 18.8	-3.9 (-21.9, 12.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-4.7 (-26.6, 17.2)
ATP Day 360	n=12 35.4 (24.33) 12.5 39.06 56.3	n=12 -5.7 (16.47) -18.8 -7.81 9.4	0.6318 -3.9 (-14.1, 6.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.15. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Family
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 49.7 (26.98) 46.9 50.00 68.8	-	-
OP Day 180	n=10 45.6 (22.16) 25.0 45.31 53.1	n=10 -4.1 (17.86) -25.0 1.56 6.3	-4.7 (-21.6, 12.2)
ATP Baseline	n=10 44.1 (22.89) 25.0 43.75 56.3	-	-
ATP Day 180	n=9 39.9 (17.66) 34.4 50.00 50.0	n=9 -6.3 (25.72) -18.8 -6.25 18.8	-0.4 (-28.1, 27.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-0.6 (-32.9, 31.8)
ATP Day 360	n=9 42.7 (21.59) 25.0 50.00 59.4	n=9 -3.5 (17.85) -15.6 -6.25 12.5	0.9697 -0.3 (-19.8, 19.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.15. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Family
 Modified Intent-to-Treat Set

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Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 20.8 (7.86) 12.5 21.88 28.1	-	-
OP Day 180	n=2 31.3 (8.84) 25.0 31.25 37.5	n=2 14.1 (15.47) 3.1 14.06 25.0	8.6 (-3.8, 21.0)
ATP Baseline	n=3 26.0 (14.09) 12.5 25.00 40.6	-	-
ATP Day 180	n=2 18.8 (26.52) 0.0 18.75 37.5	n=2 -14.1 (15.47) -25.0 -14.06 -3.1	-6.3 (-24.0, 11.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-20.3 (-38.1, -2.6) 0.0290
ATP Day 360	n=3 13.5 (20.81) 0.0 3.13 37.5	n=3 -12.5 (11.27) -25.0 -9.38 -3.1	-11.7 (-24.2, 0.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.16. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Feelings.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 44.7 (26.13) 37.5 50.00 56.3	-	-
OP Day 180	n=12 44.0 (23.14) 20.3 45.31 60.9	n=12 0.3 (21.55) -14.1 0.00 15.6	-0.8 (-18.8, 17.2)
ATP Baseline	n=13 37.0 (25.41) 15.6 34.38 59.4	-	-
ATP Day 180	n=11 25.6 (21.51) 3.1 21.88 50.0	n=11 -11.4 (20.22) -15.6 -6.25 0.0	-8.6 (-28.1, 3.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-11.7 (-32.8, 9.4)
ATP Day 360	n=12 23.7 (25.19) 3.1 12.50 50.0	n=12 -14.3 (15.57) -23.4 -10.94 -3.1	0.2881 -13.3 (-28.1, -6.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.16. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Feelings
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 43.4 (29.88) 18.8 51.56 56.3	-	-
OP Day 180	n=10 44.4 (23.70) 18.8 45.31 59.4	n=10 0.9 (22.05) -9.4 0.00 15.6	0.8 (-20.5, 22.1)
ATP Baseline	n=10 39.4 (26.61) 15.6 37.50 59.4	-	-
ATP Day 180	n=9 28.1 (22.53) 15.6 21.88 50.0	n=9 -12.8 (22.18) -15.6 -6.25 0.0	-8.3 (-32.3, 15.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-14.1 (-44.9, 16.6)
ATP Day 360	n=9 30.2 (26.01) 6.3 21.88 53.1	n=9 -10.8 (9.13) -15.6 -9.38 -6.3	0.3298 -13.5 (-21.1, -5.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.16. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Feelings
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 49.0 (6.51) 43.8 46.88 56.3	-	-
OP Day 180	n=2 42.2 (28.73) 21.9 42.19 62.5	n=2 -3.1 (26.52) -21.9 -3.13 15.6	-8.6 (-29.9, 12.7)
ATP Baseline	n=3 29.2 (23.87) 3.1 34.38 50.0	-	-
ATP Day 180	n=2 14.1 (15.47) 3.1 14.06 25.0	n=2 -4.7 (6.63) -9.4 -4.69 0.0	-22.7 (-37.8, -7.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-5.1 (-46.8, 36.7)
ATP Day 360	n=3 4.2 (4.77) 0.0 3.13 9.4	n=3 -25.0 (27.78) -46.9 -34.38 6.3	0.7919 -27.3 (-57.5, 2.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.17. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Friends.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 33.7 (19.19) 18.8 31.25 50.0	-	-
OP Day 180	n=12 39.6 (23.74) 25.0 40.63 56.3	n=12 7.8 (14.63) 0.0 9.38 12.5	10.9 (-6.2, 25.0)
ATP Baseline	n=13 47.1 (31.73) 25.0 50.00 62.5	-	-
ATP Day 180	n=11 48.3 (29.19) 25.0 50.00 56.3	n=11 -4.0 (23.26) -18.8 -6.25 12.5	-4.7 (-21.9, 12.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-14.1 (-40.6, 12.5)
ATP Day 360	n=12 43.2 (27.50) 18.8 43.75 59.4	n=12 -5.7 (25.49) -18.8 3.13 12.5	0.2461 -4.7 (-31.2, 15.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.17. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Friends
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 34.4 (19.38) 18.8 37.50 50.0	-	-
OP Day 180	n=10 45.0 (21.41) 25.0 43.75 62.5	n=10 10.6 (11.43) 0.0 9.38 12.5	12.5 (1.8, 23.2)
ATP Baseline	n=10 50.0 (25.34) 25.0 53.13 62.5	-	-
ATP Day 180	n=9 46.5 (23.82) 31.3 50.00 50.0	n=9 -6.3 (25.19) -18.8 -6.25 6.3	-7.8 (-29.4, 13.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-19.3 (-49.6, 11.0) 0.1858
ATP Day 360	n=9 42.4 (22.05) 25.0 50.00 56.3	n=9 -10.4 (27.78) -25.0 0.00 12.5	-9.6 (-36.8, 17.7)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.17. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Friends
 Modified Intent-to-Treat Set

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Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 31.3 (22.53) 12.5 25.00 56.3	-	-
OP Day 180	n=2 12.5 (17.68) 0.0 12.50 25.0	n=2 -6.3 (26.52) -25.0 -6.25 12.5	-20.3 (-52.3, 11.7)
ATP Baseline	n=3 37.5 (54.49) 0.0 12.50 100.0	-	-
ATP Day 180	n=2 56.3 (61.87) 12.5 56.25 100.0	n=2 6.3 (8.84) 0.0 6.25 12.5	28.1 (10.4, 45.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	37.5 (30.4, 44.6) <.0001
ATP Day 360	n=3 45.8 (46.91) 18.8 18.75 100.0	n=3 8.3 (9.55) 0.0 6.25 18.8	12.5 (5.4, 19.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.18. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Future.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 39.9 (14.33) 37.5 37.50 50.0	-	-
OP Day 180	n=12 38.0 (19.67) 28.1 43.75 50.0	n=12 -1.0 (17.44) -9.4 -3.13 3.1	-7.8 (-18.8, 18.8)
ATP Baseline	n=13 35.6 (17.56) 25.0 37.50 50.0	-	-
ATP Day 180	n=11 35.2 (21.88) 25.0 37.50 50.0	n=11 0.0 (12.18) -6.3 0.00 0.0	1.6 (-25.0, 18.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	4.7 (-28.1, 31.2)
ATP Day 360	n=12 34.4 (17.37) 18.8 37.50 50.0	n=12 -1.0 (13.01) -6.3 0.00 6.3	0.8438 -1.6 (-12.5, 9.4)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.18. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Future
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 40.6 (15.38) 37.5 37.50 50.0	-	-
OP Day 180	n=10 40.6 (16.99) 31.3 43.75 50.0	n=10 0.0 (16.93) -6.3 -3.13 0.0	-6.3 (-24.0, 11.5)
ATP Baseline	n=10 36.9 (14.86) 25.0 37.50 50.0	-	-
ATP Day 180	n=9 36.8 (19.63) 25.0 37.50 50.0	n=9 0.0 (13.62) -6.3 0.00 0.0	-0.3 (-24.9, 24.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	1.9 (-33.1, 36.9)
ATP Day 360	n=9 35.4 (16.83) 18.8 37.50 50.0	n=9 -1.4 (14.91) -6.3 0.00 6.3	0.9053 -2.6 (-18.4, 13.2)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.18. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Future
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 37.5 (12.50) 25.0 37.50 50.0	-	-
OP Day 180	n=2 25.0 (35.36) 0.0 25.00 50.0	n=2 -6.3 (26.52) -25.0 -6.25 12.5	-15.6 (-36.9, 5.7)
ATP Baseline	n=3 31.3 (28.64) 0.0 37.50 56.3	-	-
ATP Day 180	n=2 28.1 (39.77) 0.0 28.13 56.3	n=2 0.0 (0.00) 0.0 0.00 0.0	-9.4 (-9.4, -9.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	13.3 (-13.4, 39.9)
ATP Day 360	n=3 31.3 (22.53) 6.3 37.50 50.0	n=3 0.0 (6.25) -6.3 0.00 6.3	0.2928 0.0 (-7.1, 7.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.19. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Other People.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 30.4 (24.20) 8.3 29.17 54.2	-	-
OP Day 180	n=12 35.1 (21.43) 12.5 39.58 54.2	n=12 2.8 (16.51) -4.2 0.00 2.1	-2.1 (-10.4, 18.8)
ATP Baseline	n=13 32.6 (22.57) 8.3 40.00 50.0	-	-
ATP Day 180	n=11 28.0 (20.50) 12.5 25.00 50.0	n=11 -4.0 (11.41) -16.7 0.00 4.2	-6.3 (-14.6, 1.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-6.3 (-27.1, 7.1)
ATP Day 360	n=12 25.3 (18.92) 10.4 22.92 39.6	n=12 -9.2 (15.44) -18.8 -4.17 2.1	0.3740 -9.4 (-20.8, 0.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.19. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Other People
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 35.0 (25.47) 8.3 47.92 58.3	-	-
OP Day 180	n=10 35.4 (20.53) 12.5 39.58 54.2	n=10 0.4 (15.77) -4.2 -2.08 0.0	-4.2 (-21.9, 13.6)
ATP Baseline	n=10 34.8 (20.47) 12.5 42.92 50.0	-	-
ATP Day 180	n=9 31.5 (21.26) 12.5 33.33 50.0	n=9 -6.3 (11.37) -16.7 0.00 1.7	-7.4 (-20.3, 5.4)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-3.0 (-25.7, 19.7)
ATP Day 360	n=9 29.2 (19.98) 12.5 25.00 45.8	n=9 -8.6 (13.94) -16.7 -4.17 0.0	0.7769 -6.7 (-20.0, 6.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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 TABLE 6.1.19. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Other People
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 15.3 (12.03) 8.3 8.33 29.2	-	-
OP Day 180	n=2 33.3 (35.36) 8.3 33.33 58.3	n=2 14.6 (20.62) 0.0 14.58 29.2	27.1 (24.7, 29.5)
ATP Baseline	n=3 25.0 (32.54) 4.2 8.33 62.5	-	-
ATP Day 180	n=2 12.5 (0.00) 12.5 12.50 12.5	n=2 6.3 (2.95) 4.2 6.25 8.3	NE
Treatment difference ATP Day 180 vs. OP Day 180	-	-	NE
ATP Day 360	n=3 13.9 (10.49) 4.2 12.50 25.0	n=3 -11.1 (22.95) -37.5 0.00 4.2	-16.7 (-40.4, 7.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.20. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Perceived Support.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 43.3 (26.33) 31.3 37.50 56.3	-	-
OP Day 180	n=12 41.1 (19.30) 28.1 50.00 56.3	n=12 2.6 (19.85) -12.5 3.13 15.6	1.6 (-15.6, 18.8)
ATP Baseline	n=13 49.0 (27.10) 25.0 50.00 56.3	-	-
ATP Day 180	n=11 52.8 (24.10) 37.5 43.75 50.0	n=11 2.3 (26.11) -12.5 6.25 18.8	4.7 (-18.8, 18.8)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-1.6 (-25.0, 25.0)
ATP Day 360	n=12 47.4 (31.58) 18.8 50.00 75.0	n=12 -3.6 (23.00) -21.9 -6.25 15.6	0.8027 -4.7 (-21.9, 12.5)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.20. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Perceived Support
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 40.0 (22.86) 25.0 40.63 56.3	-	-
OP Day 180	n=10 43.8 (17.43) 31.3 50.00 56.3	n=10 3.8 (19.14) -6.3 3.13 12.5	3.1 (-14.6, 20.9)
ATP Baseline	n=10 46.3 (23.97) 25.0 46.88 56.3	-	-
ATP Day 180	n=9 49.3 (20.12) 37.5 43.75 50.0	n=9 0.7 (28.55) -12.5 6.25 18.8	6.3 (-20.5, 33.0)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	0.9 (-31.8, 33.6)
ATP Day 360	n=9 41.7 (30.46) 18.8 37.50 75.0	n=9 -6.9 (25.85) -25.0 -12.50 18.8	0.9517 -10.7 (-31.2, 9.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.20. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Perceived Support
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 54.2 (39.69) 31.3 31.25 100.0	-	-
OP Day 180	n=2 28.1 (30.94) 6.3 28.13 50.0	n=2 -3.1 (30.94) -25.0 -3.13 18.8	-25.8 (-77.3, 25.7)
ATP Baseline	n=3 58.3 (40.67) 18.8 56.25 100.0	-	-
ATP Day 180	n=2 68.8 (44.19) 37.5 68.75 100.0	n=2 9.4 (13.26) 0.0 9.38 18.8	15.6 (12.1, 19.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	38.3 (-6.1, 82.7)
ATP Day 360	n=3 64.6 (34.42) 31.3 62.50 100.0	n=3 6.3 (6.25) 0.0 6.25 12.5	0.0837 9.4 (5.8, 12.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.21. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Physical Health.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 57.4 (17.34) 46.4 60.71 71.4	-	-
OP Day 180	n=12 46.7 (19.82) 33.9 50.00 62.5	n=12 -12.5 (26.31) -25.0 -8.93 5.4	-11.6 (-33.9, 5.4)
ATP Baseline	n=13 48.1 (19.21) 35.7 50.00 60.7	-	-
ATP Day 180	n=11 22.1 (21.24) 0.0 21.43 39.3	n=11 -25.3 (19.59) -32.1 -17.86 -14.3	-21.4 (-42.9, -3.6)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-5.4 (-26.8, 10.7) 0.5059
ATP Day 360	n=12 23.5 (22.71) 3.6 21.43 35.7	n=12 -24.4 (18.39) -37.5 -19.64 -12.5	-21.4 (-35.7, -8.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.21. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Physical Health
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 57.5 (15.92) 46.4 62.50 71.4	-	-
OP Day 180	n=10 43.6 (20.19) 28.6 50.00 60.7	n=10 -13.9 (26.70) -21.4 -8.93 3.6	-12.5 (-36.9, 11.9)
ATP Baseline	n=10 51.4 (19.14) 35.7 50.00 60.7	-	-
ATP Day 180	n=9 22.2 (22.00) 0.0 21.43 35.7	n=9 -29.4 (19.13) -32.1 -25.00 -14.3	-23.9 (-40.0, -7.7)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-13.2 (-34.8, 8.4) 0.2043
ATP Day 360	n=9 27.0 (24.69) 3.6 28.57 39.3	n=9 -24.6 (19.19) -28.6 -21.43 -14.3	-23.4 (-38.9, -7.8)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.21. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Physical Health
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 57.1 (25.75) 35.7 50.00 85.7	-	-
OP Day 180	n=2 62.5 (7.58) 57.1 62.50 67.9	n=2 -5.4 (32.83) -28.6 -5.36 17.9	8.5 (-23.0, 39.9)
ATP Baseline	n=3 36.9 (17.98) 17.9 39.29 53.6	-	-
ATP Day 180	n=2 21.4 (25.25) 3.6 21.43 39.3	n=2 -7.1 (10.10) -14.3 -7.14 0.0	-23.2 (-33.4, -13.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-25.9 (-70.6, 18.8) 0.2255
ATP Day 360	n=3 13.1 (13.52) 3.6 7.14 28.6	n=3 -23.8 (19.67) -46.4 -14.29 -10.7	-21.4 (-41.7, -1.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
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 TABLE 6.1.22. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Relationship.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 42.3 (28.66) 25.0 50.00 50.0	-	-
OP Day 180	n=12 40.6 (38.48) 0.0 43.75 68.8	n=12 -4.2 (36.67) -37.5 0.00 25.0	-3.1 (-43.8, 37.5)
ATP Baseline	n=13 25.0 (34.23) 0.0 0.00 50.0	-	-
ATP Day 180	n=11 29.5 (26.38) 0.0 50.00 50.0	n=11 0.0 (23.05) 0.0 0.00 12.5	-3.1 (-50.0, 37.5)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	3.1 (-31.2, 43.8)
ATP Day 360	n=12 14.6 (24.33) 0.0 0.00 31.3	n=12 -12.5 (36.15) 0.0 0.00 0.0	0.9062 -56.2 (-100.0, 25.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.22. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Relationship
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 45.0 (31.29) 25.0 50.00 62.5	-	-
OP Day 180	n=10 48.8 (37.01) 12.5 50.00 75.0	n=10 3.8 (34.89) -25.0 0.00 37.5	6.3 (-43.5, 56.0)
ATP Baseline	n=10 32.5 (35.94) 0.0 25.00 50.0	-	-
ATP Day 180	n=9 36.1 (24.56) 12.5 50.00 50.0	n=9 0.0 (25.77) 0.0 0.00 12.5	5.5 (-40.0, 51.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-6.3 (-57.5, 45.0)
ATP Day 360	n=9 19.4 (26.60) 0.0 0.00 50.0	n=9 -16.7 (41.46) 0.0 0.00 0.0	0.7914 -47.3 (-123.6, 29.1)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
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 TABLE 6.1.22. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Relationship
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 33.3 (19.09) 12.5 37.50 50.0	-	-
OP Day 180	n=2 0.0 (0.00) 0.0 0.00 0.0	n=2 -43.8 (8.84) -50.0 -43.75 -37.5	NE
ATP Baseline	n=3 0.0 (0.00) 0.0 0.00 0.0	-	-
ATP Day 180	n=2 0.0 (0.00) 0.0 0.00 0.0	n=2 0.0 (0.00) 0.0 0.00 0.0	NE
Treatment difference ATP Day 180 vs. OP Day 180	-	-	NE
ATP Day 360	n=3 0.0 (0.00) 0.0 0.00 0.0	n=3 0.0 (0.00) 0.0 0.00 0.0	NE

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.23. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Sports and School.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 53.2 (17.64) 44.4 50.00 61.1	-	-
OP Day 180	n=12 53.2 (22.31) 37.5 51.39 68.1	n=12 -0.9 (22.67) -12.5 -1.39 9.7	-1.4 (-18.1, 13.9)
ATP Baseline	n=12 52.1 (17.61) 41.7 55.56 61.1	-	-
ATP Day 180	n=11 41.9 (20.28) 22.2 41.67 61.1	n=10 -7.2 (15.67) -8.3 -5.56 2.8	-4.9 (-18.1, 4.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-4.9 (-11.1, 2.8)
ATP Day 360	n=12 38.9 (16.62) 26.4 34.72 50.0	n=11 -12.1 (15.38) -16.7 -5.56 -2.8	0.1982 -13.2 (-26.4, -5.6)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.23. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Sports and School
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 54.4 (19.91) 44.4 48.61 72.2	-	-
OP Day 180	n=10 47.5 (17.78) 33.3 48.61 55.6	n=10 -6.9 (18.30) -16.7 -4.17 2.8	-6.9 (-25.1, 11.2)
ATP Baseline	n=9 50.0 (16.67) 50.0 55.56 55.6	-	-
ATP Day 180	n=9 40.4 (19.79) 22.2 41.67 58.3	n=8 -9.0 (16.98) -16.7 -6.94 2.8	-3.3 (-21.5, 14.9)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	1.7 (-13.5, 17.0)
ATP Day 360	n=9 37.0 (15.71) 22.2 36.11 50.0	n=8 -11.5 (11.45) -15.3 -8.33 -4.2	0.8029 -8.9 (-26.1, 8.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.23. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Sports and School
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 49.1 (6.99) 41.7 50.00 55.6	-	-
OP Day 180	n=2 81.9 (25.53) 63.9 81.94 100.0	n=2 29.2 (21.61) 13.9 29.17 44.4	34.7 (17.3, 52.1)
ATP Baseline	n=3 58.3 (22.74) 33.3 63.89 77.8	-	-
ATP Day 180	n=2 48.6 (29.46) 27.8 48.61 69.4	n=2 0.0 (7.86) -5.6 0.00 5.6	-8.7 (-28.4, 11.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-43.4 (-80.5, -6.3) 0.0262
ATP Day 360	n=3 44.4 (21.70) 30.6 33.33 69.4	n=3 -13.9 (26.79) -44.4 -2.78 5.6	-11.1 (-39.5, 17.3)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
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 Datacut: B7841005 (29APR2025)
 TABLE 6.1.24. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Treatment.
 Modified Intent-to-Treat Set

Table page 1 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Overall			
Modified Intent-to-Treat Set			
N=13			
OP Baseline	n=13 34.6 (18.42) 21.9 34.38 50.0	-	-
OP Day 180	n=12 36.2 (17.70) 21.9 32.81 51.6	n=12 1.6 (15.91) -4.7 0.00 9.4	2.3 (-12.5, 17.2)
ATP Baseline	n=13 38.7 (14.18) 34.4 43.75 46.9	-	-
ATP Day 180	n=11 30.1 (16.31) 15.6 31.25 46.9	n=11 -9.7 (17.31) -21.9 -9.38 3.1	-10.2 (-23.4, 3.1)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-8.6 (-25.0, 4.7)
ATP Day 360	n=12 31.5 (17.80) 17.2 31.25 50.0	n=12 -8.6 (12.72) -17.2 -12.50 1.6	0.1255 -10.2 (-18.8, 0.0)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
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 TABLE 6.1.24. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Treatment
 Modified Intent-to-Treat Set

Table page 2 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia A			
N=10			
OP Baseline	n=10 37.5 (19.93) 31.3 42.19 50.0	-	-
OP Day 180	n=10 37.5 (17.49) 31.3 32.81 53.1	n=10 0.0 (15.45) -3.1 0.00 6.3	0.8 (-19.6, 21.2)
ATP Baseline	n=10 40.0 (13.40) 34.4 39.06 46.9	-	-
ATP Day 180	n=9 31.6 (17.43) 21.9 34.38 46.9	n=9 -10.4 (18.68) -21.9 -9.38 0.0	-13.5 (-33.3, 6.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-10.0 (-28.9, 8.9)
ATP Day 360	n=9 33.0 (18.76) 18.8 31.25 53.1	n=9 -9.0 (12.74) -15.6 -12.50 -3.1	0.2675 -9.5 (-21.0, 1.9)

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 6.1.24. Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Treatment
 Modified Intent-to-Treat Set

Table page 3 of 3.

Subgroup, Study Visit	Actual Values (descriptive)	Change from Baseline (descriptive)	Non-parametric Change from Baseline
Disease Type			
Hemophilia B			
N=3			
OP Baseline	n=3 25.0 (8.27) 18.8 21.88 34.4	-	-
OP Day 180	n=2 29.7 (24.31) 12.5 29.69 46.9	n=2 9.4 (22.10) -6.3 9.38 25.0	2.3 (-15.4, 20.1)
ATP Baseline	n=3 34.4 (19.01) 12.5 43.75 46.9	-	-
ATP Day 180	n=2 23.4 (11.05) 15.6 23.44 31.3	n=2 -6.3 (13.26) -15.6 -6.25 3.1	-12.1 (-25.4, 1.2)
Treatment difference ATP Day 180 vs. OP Day 180	-	-	-14.4 (-45.5, 16.6)
ATP Day 360	n=3 27.1 (17.21) 15.6 18.75 46.9	n=3 -7.3 (15.42) -25.0 0.00 3.1	0.3247 -10.9 (-26.9, 5.0)

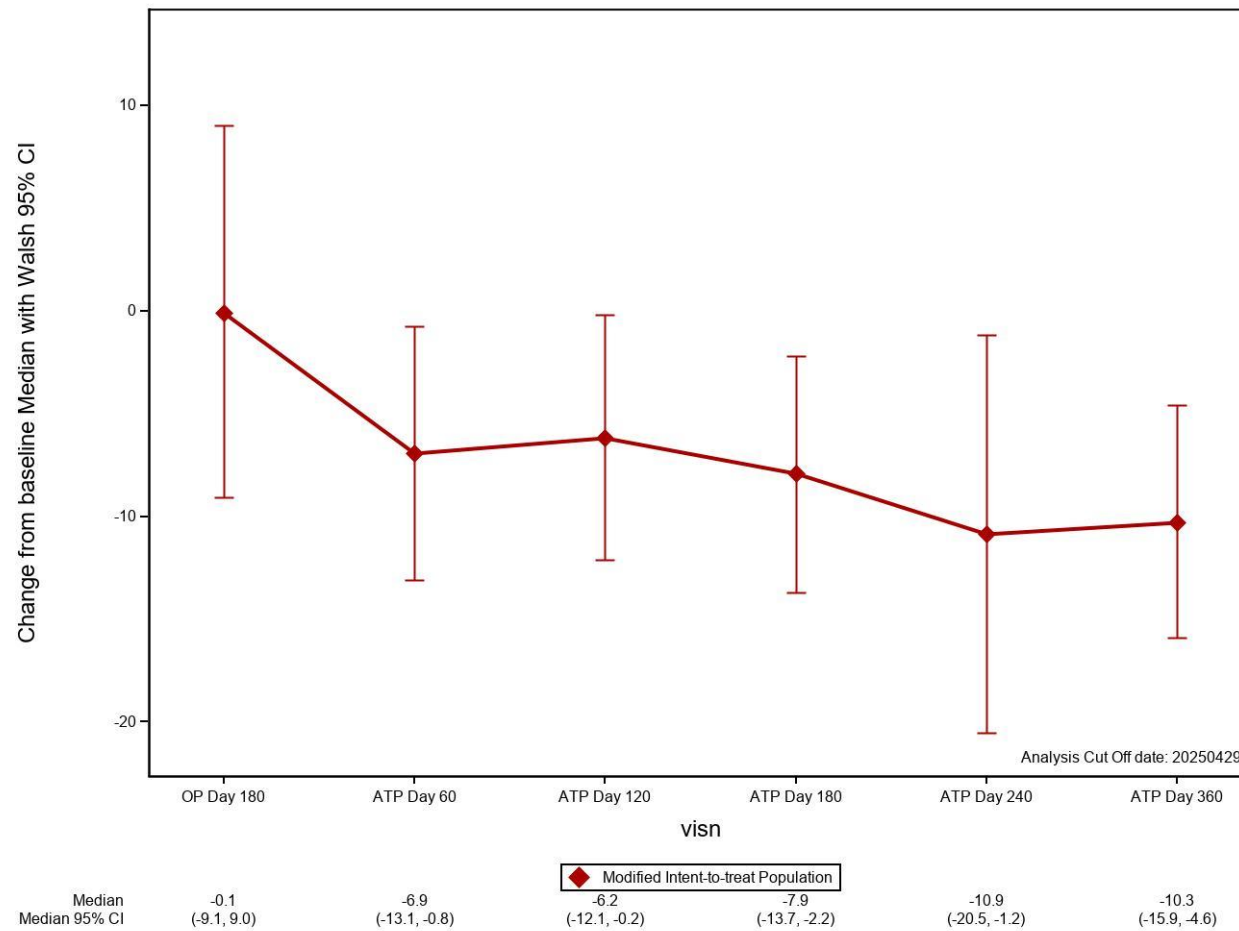
ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the Modified Intent-to-treat Set; n: number of patients with a value; NE: not estimable; OP: Observational Phase.

Descriptive analyses are presented as n, mean (standard deviation) and quartiles (Q1 |median | Q3).

Non-parametric change from baseline analyses show the median with exact confidence interval using Walsh averages and the p-value from Wilcoxon Signed Rank test for the treatment difference (ATP at 6 months vs. OP at 6 months). Missing values were imputed using multiple imputation methods based on MAR assumption. Non-estimable (NE) results can be due to excessive ties in the data, or due to insufficient observations to perform the multiple imputation.

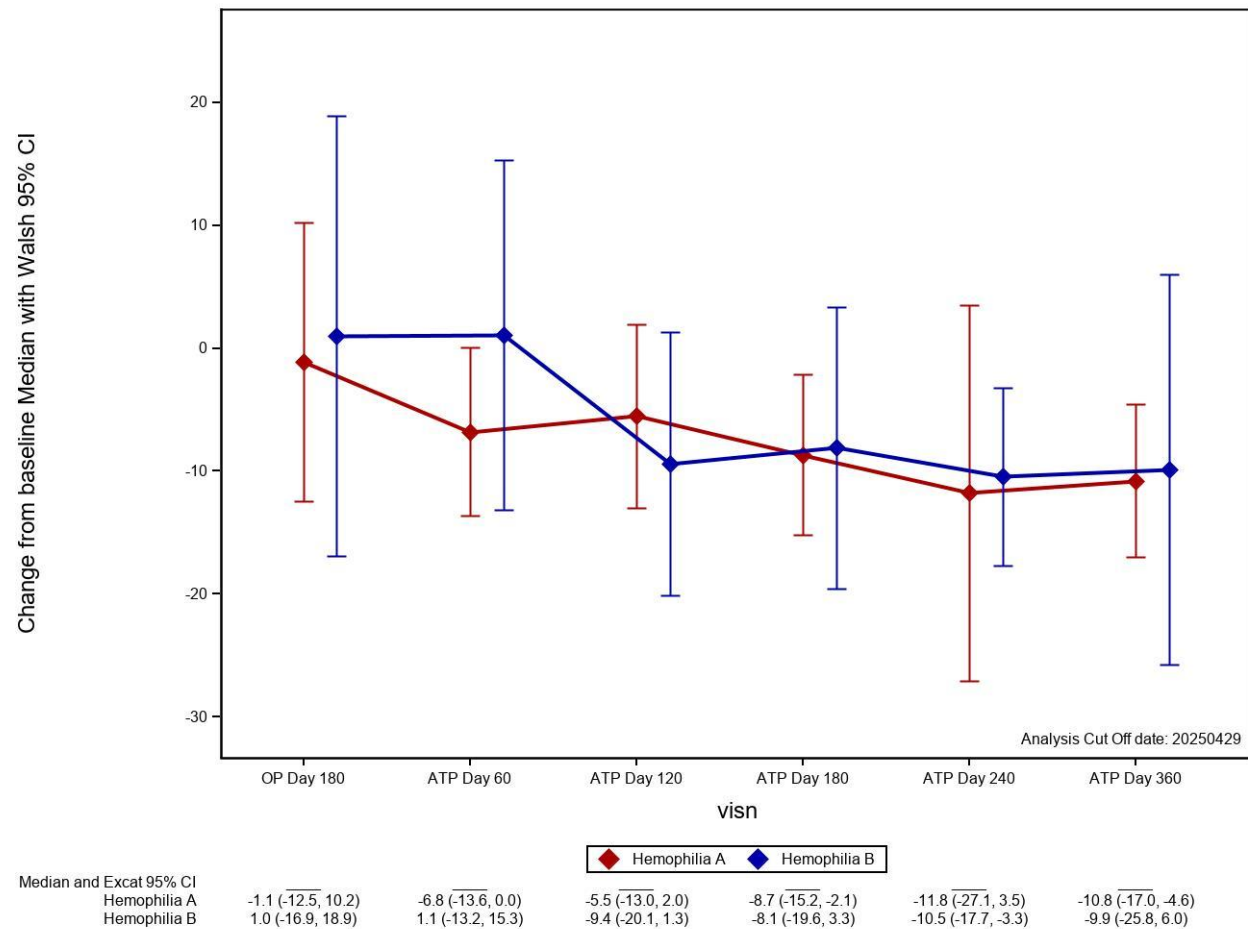
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.12.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Total Score - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



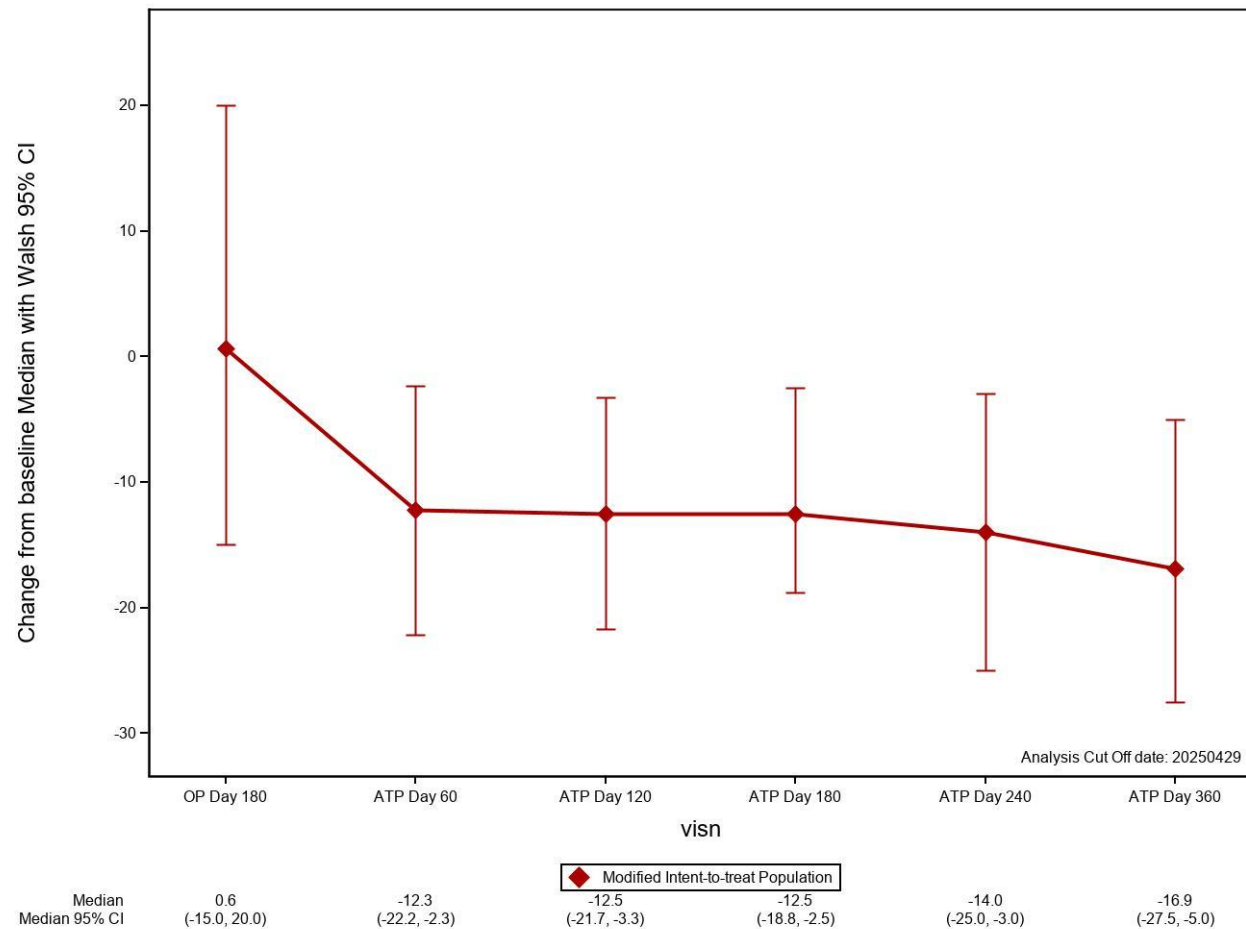
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.12.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Total Score - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



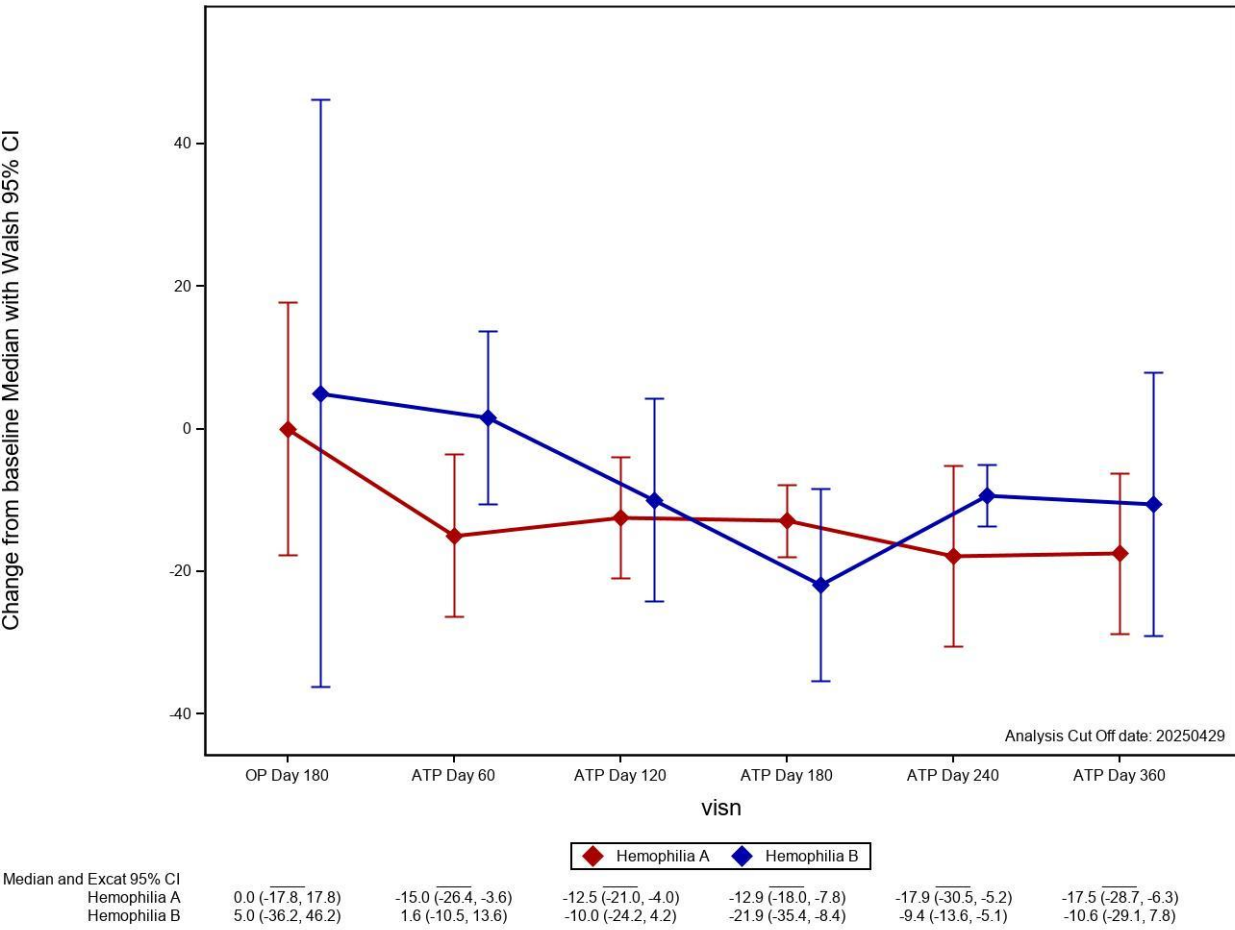
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.13.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Attitude/View - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



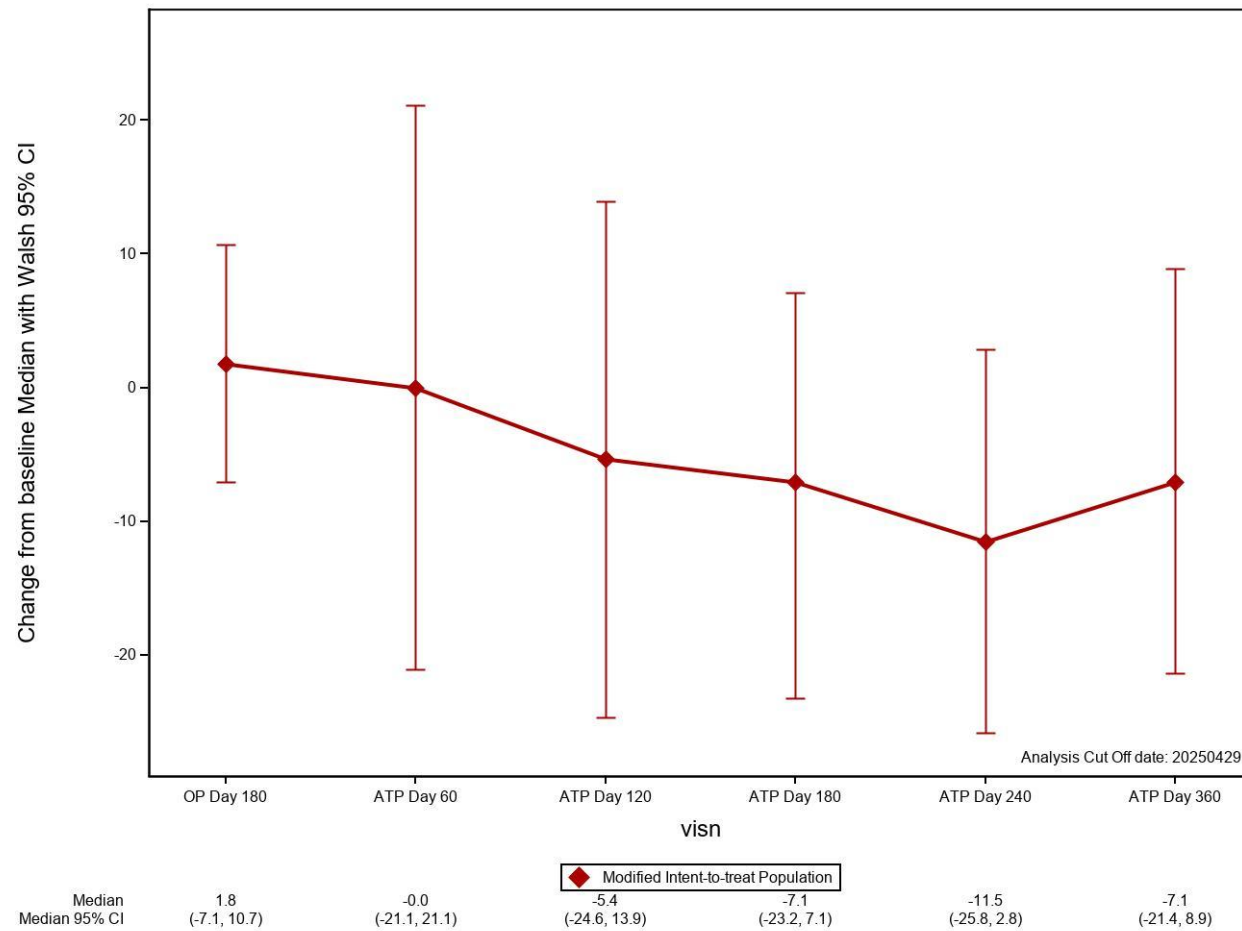
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.13.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Attitude/View - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



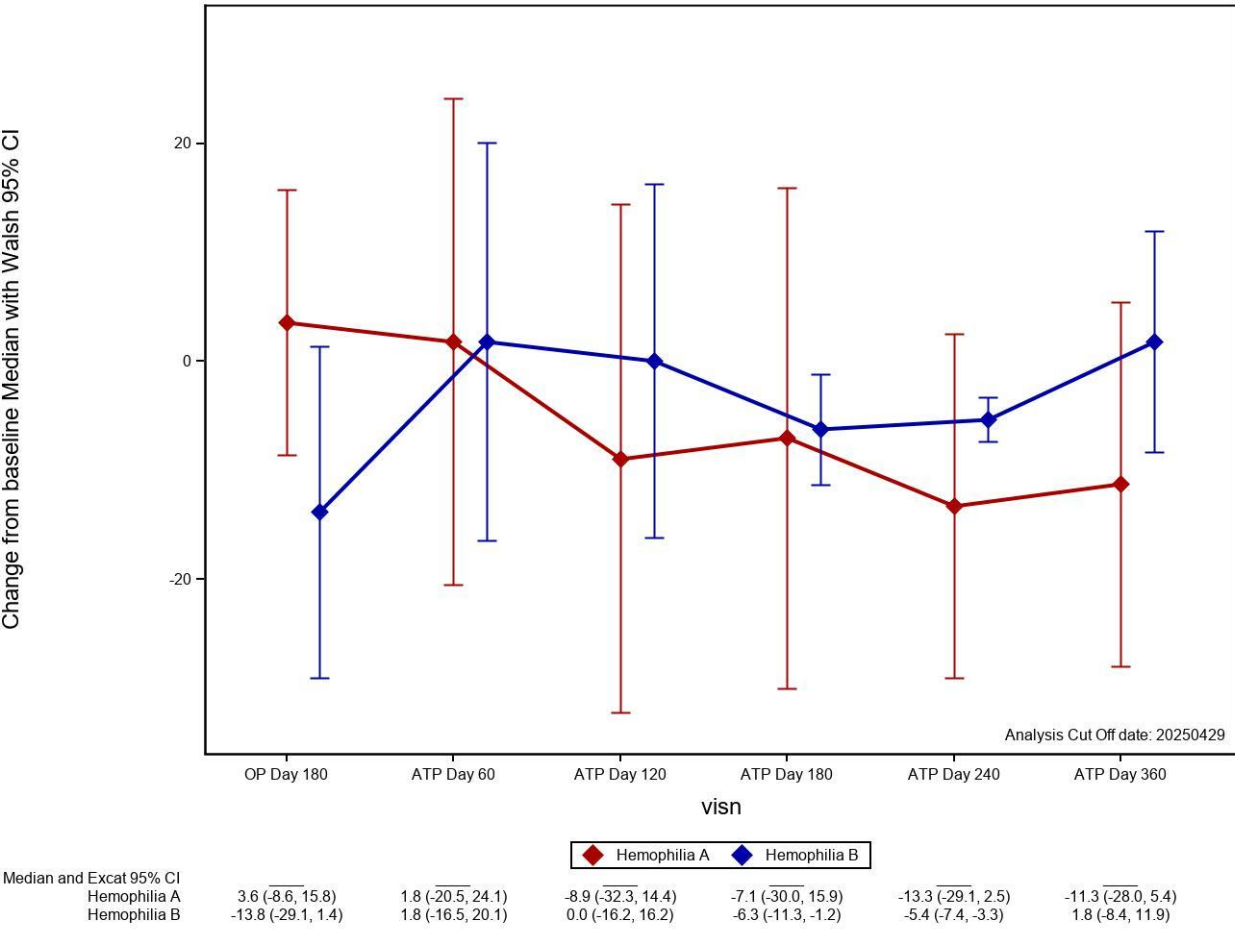
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.14.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Coping/Dealing - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



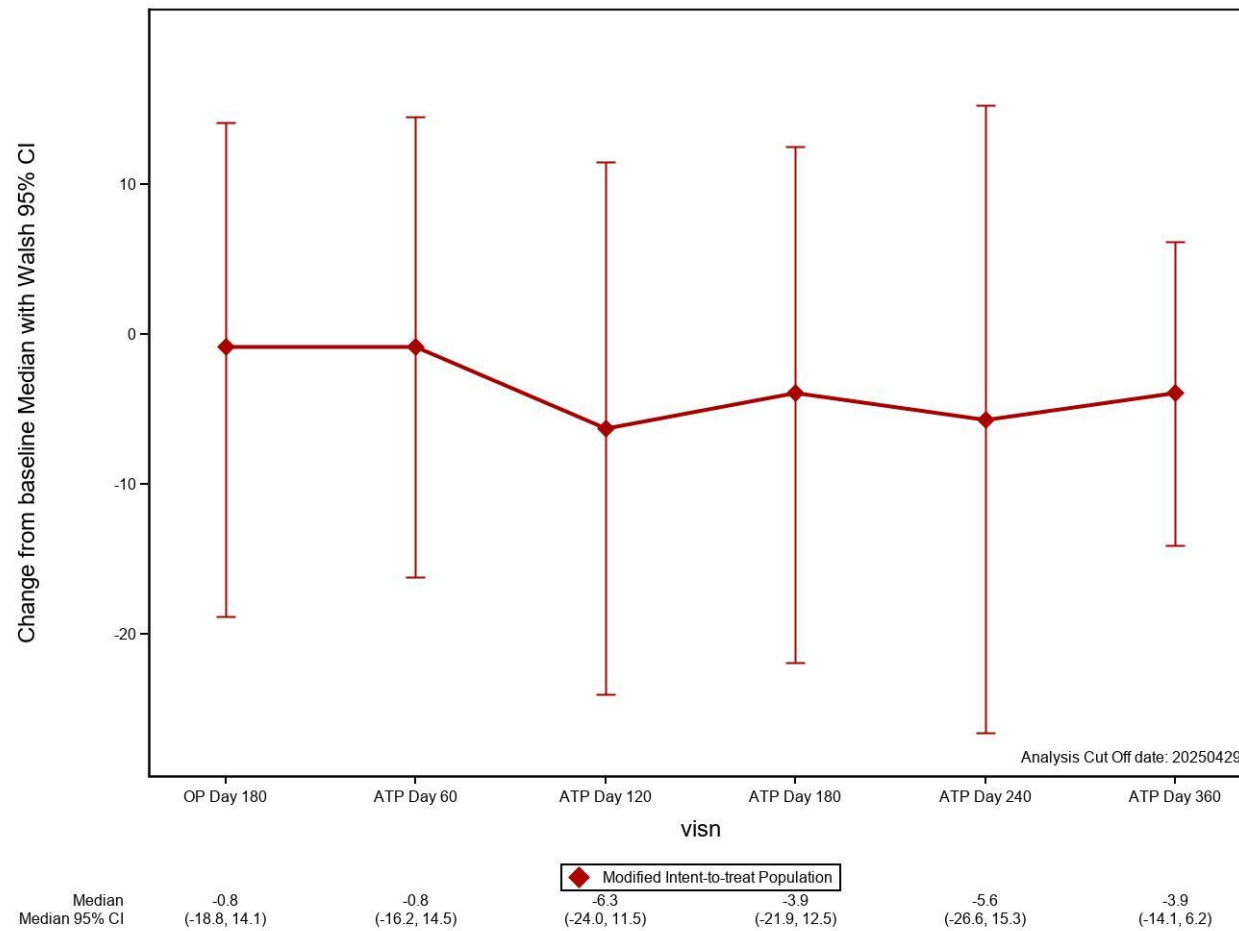
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.14.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Coping/Dealing - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



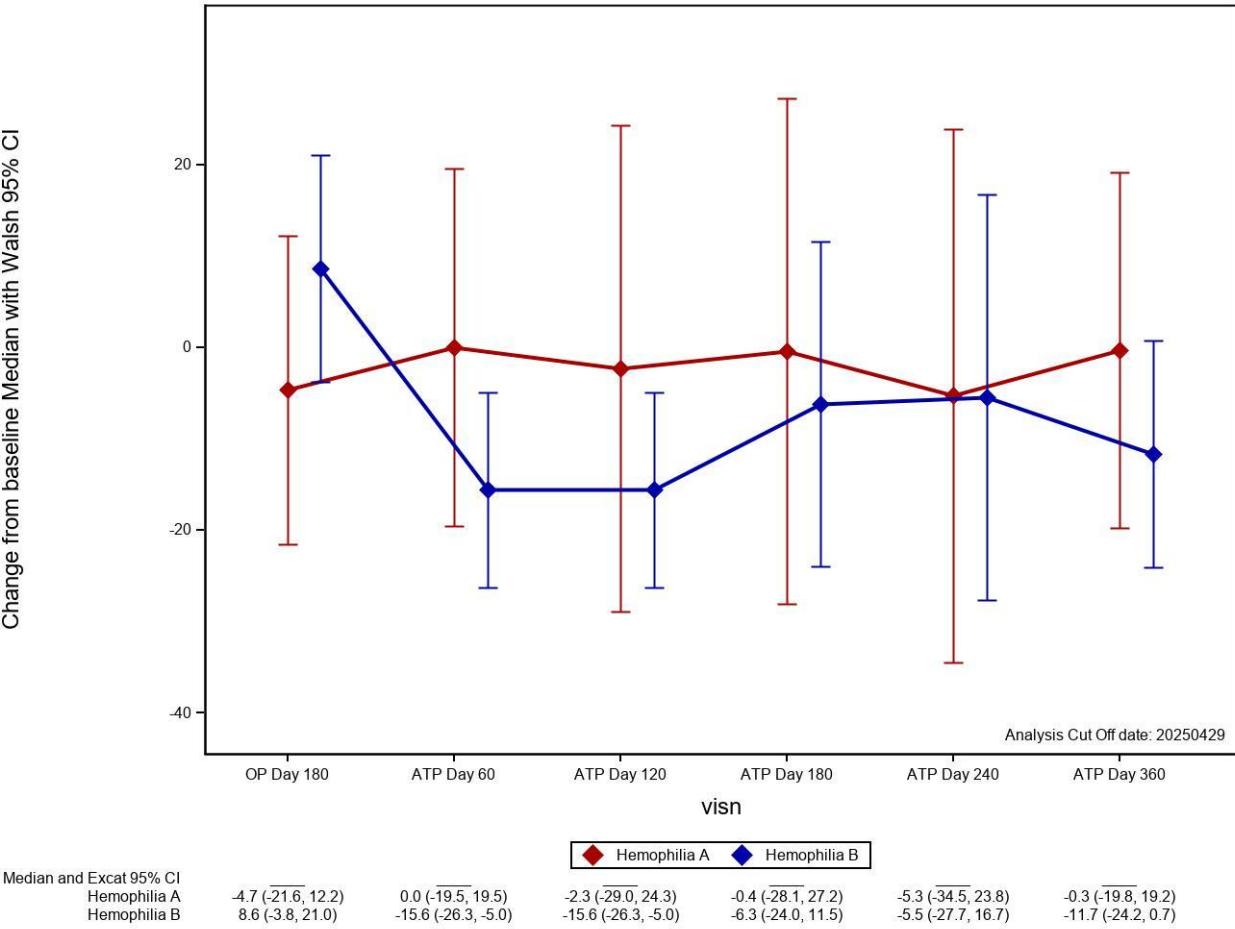
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.15.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Family - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



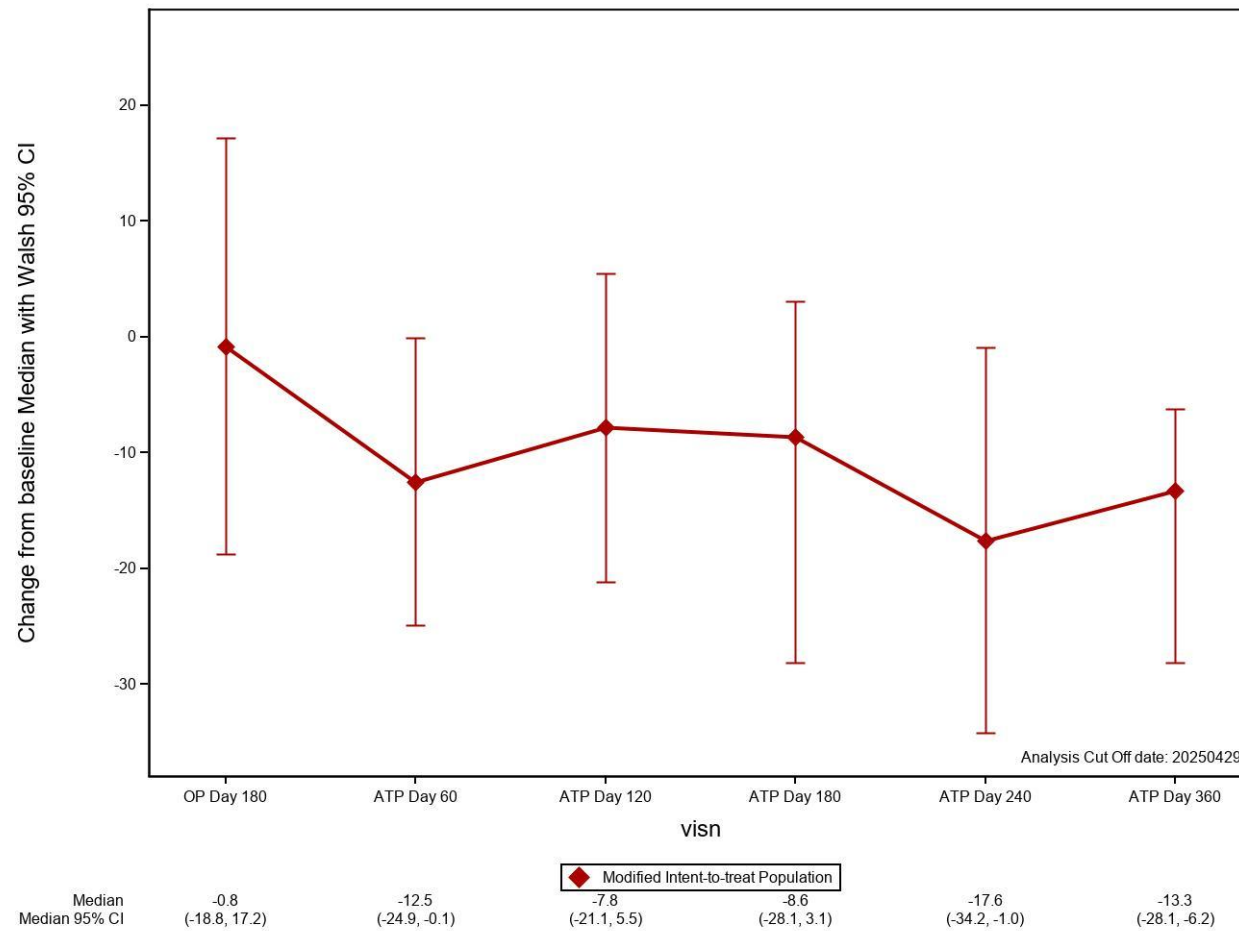
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.15.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Family - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



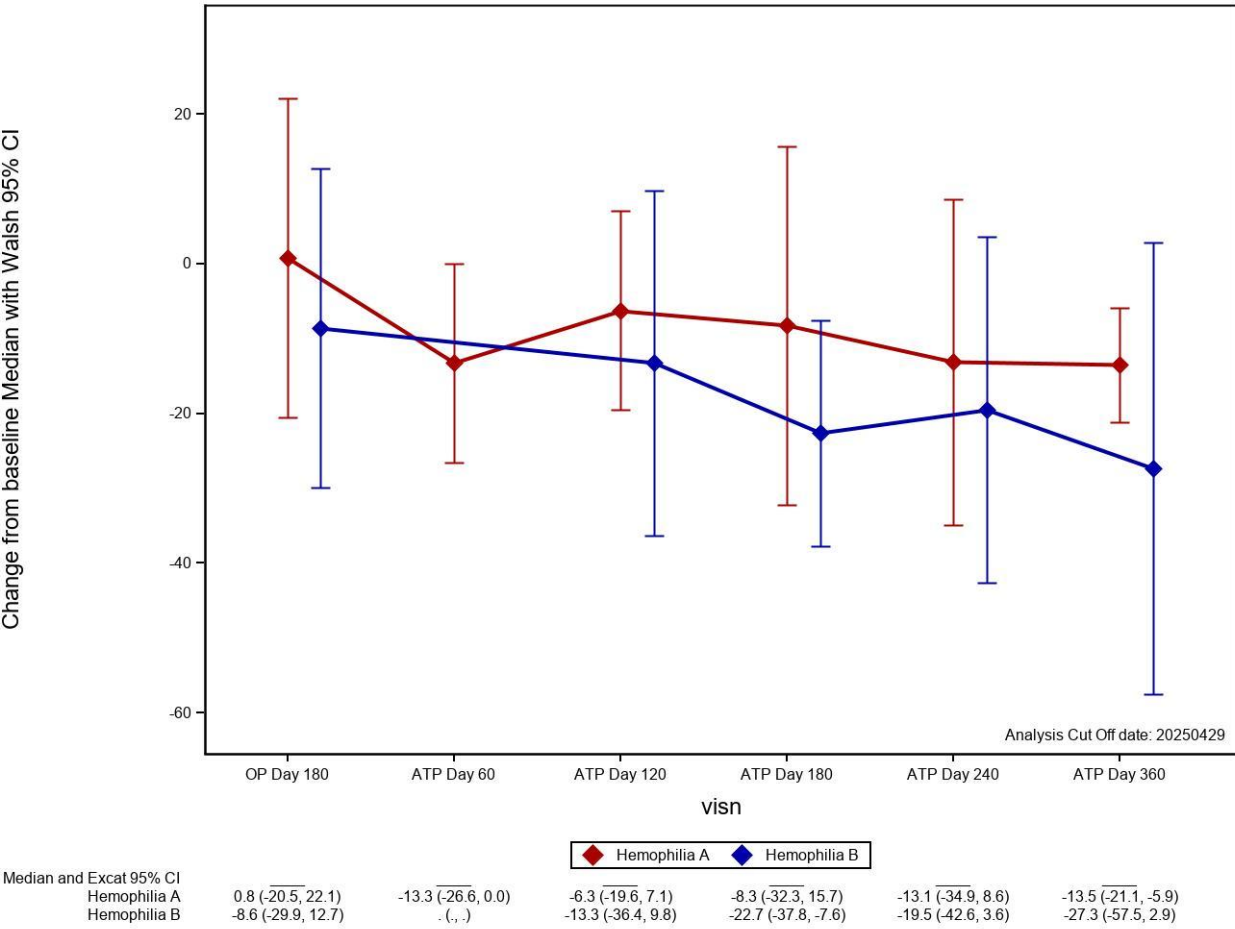
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.16.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Feelings - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



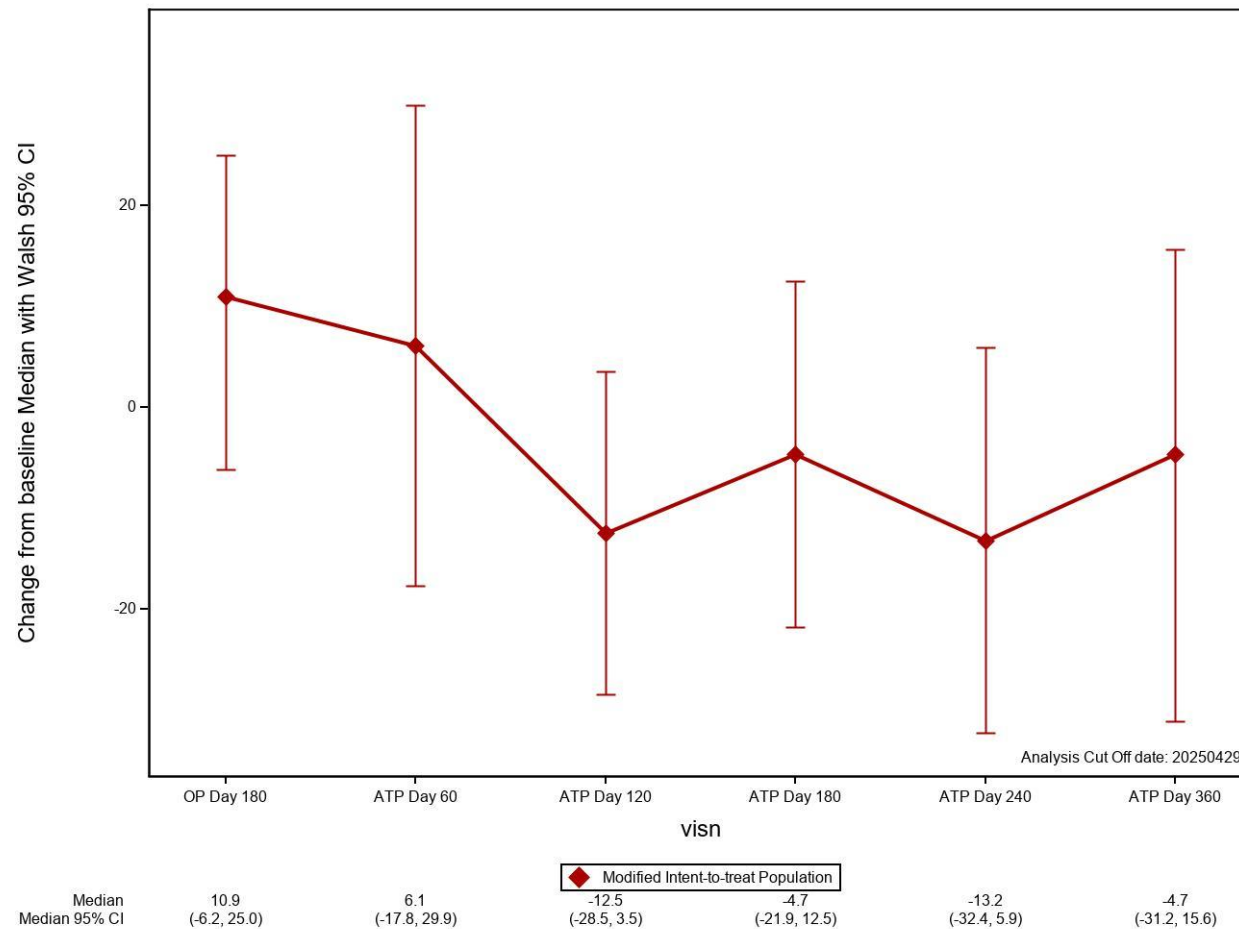
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.16.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Feelings - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



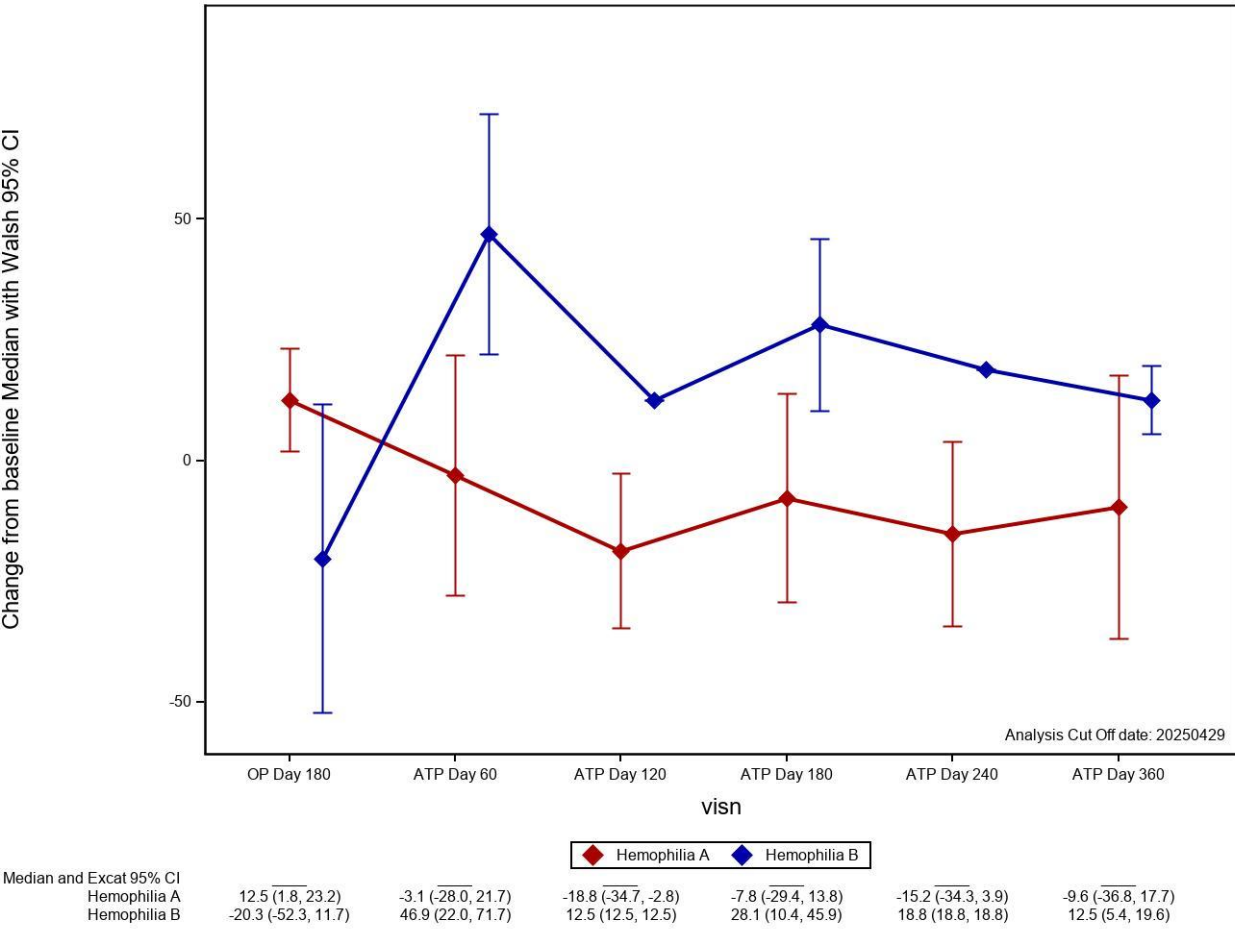
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.17.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Friends - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



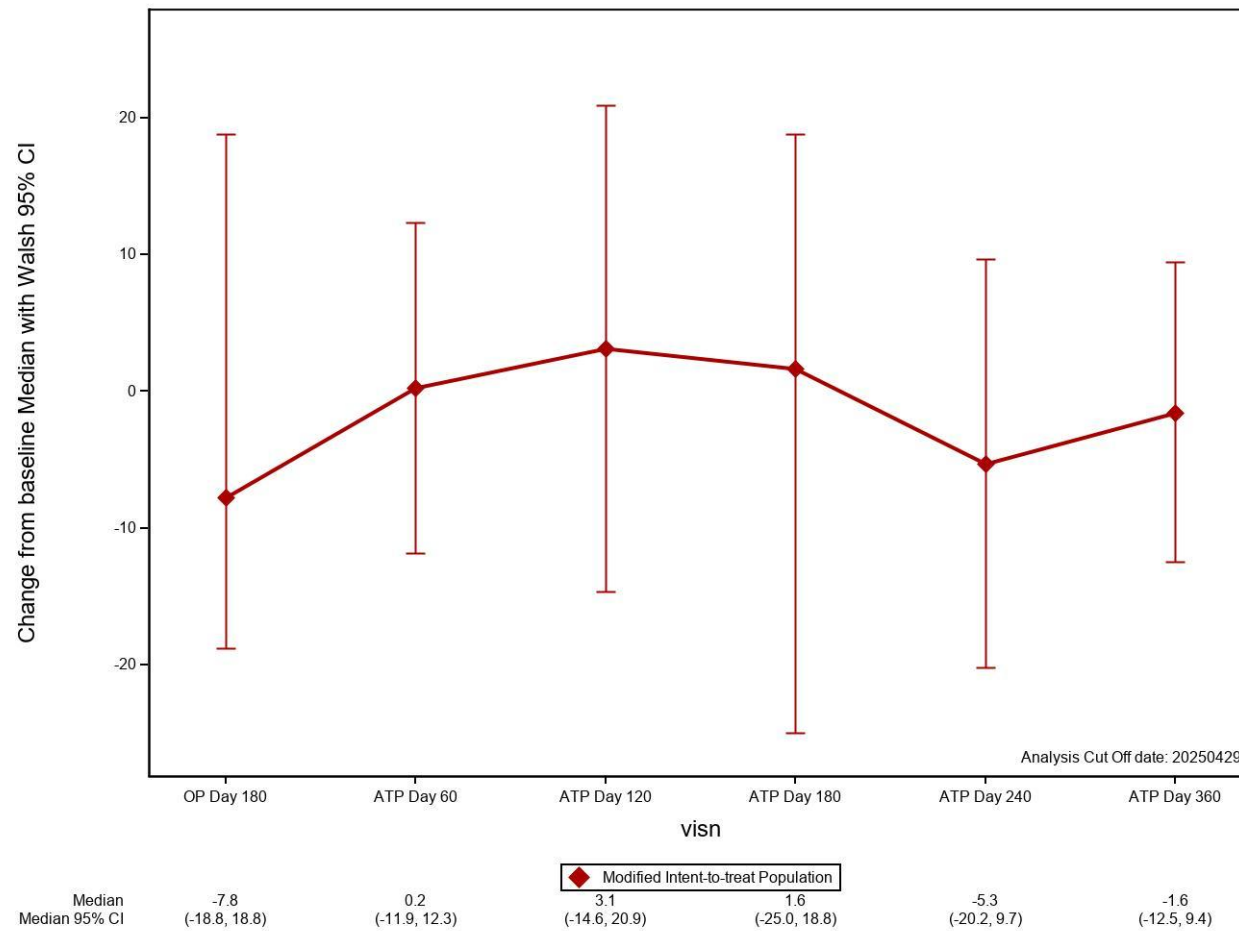
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.17.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Friends - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



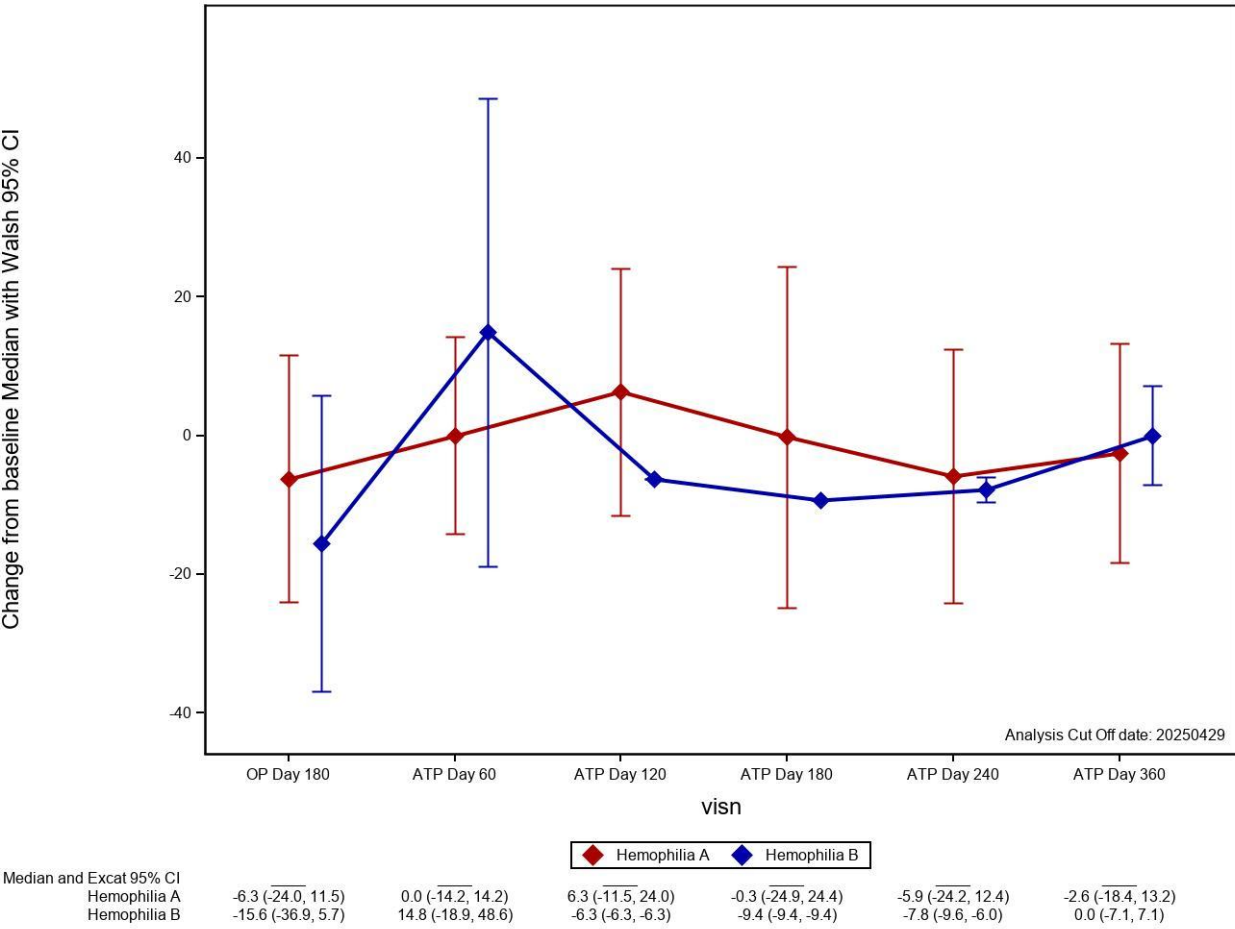
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.18.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Future - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



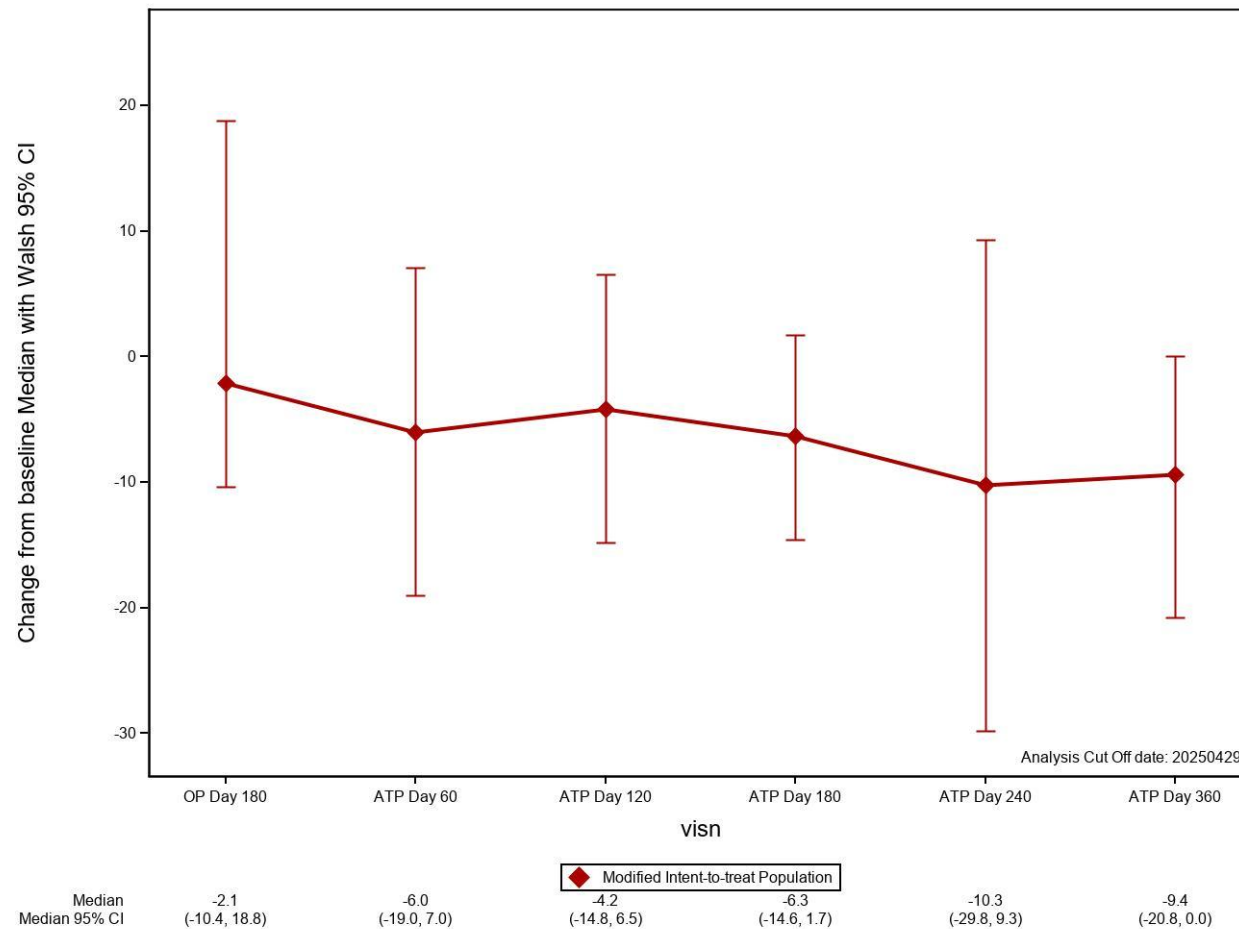
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.18.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Future - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



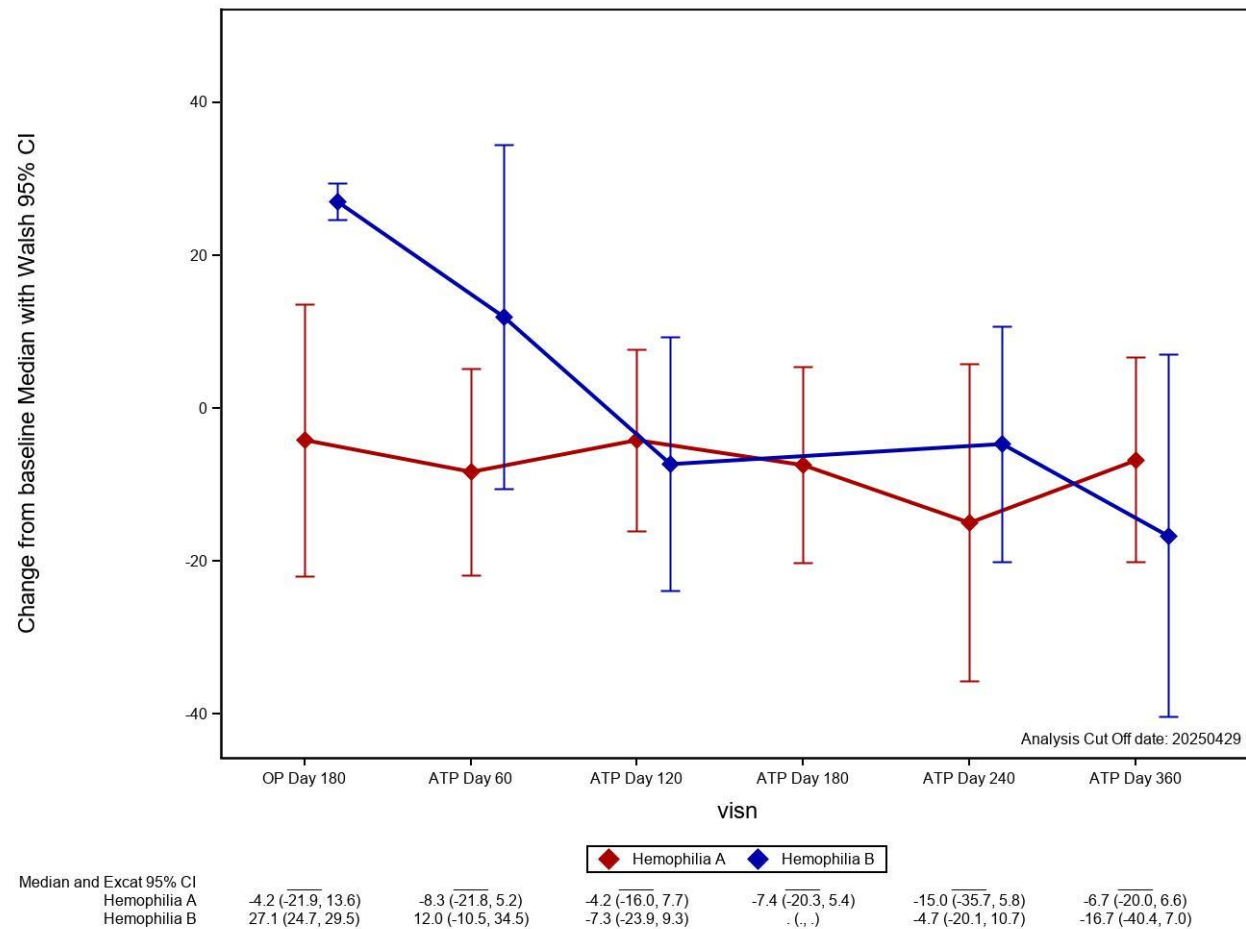
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.19.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Other People - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



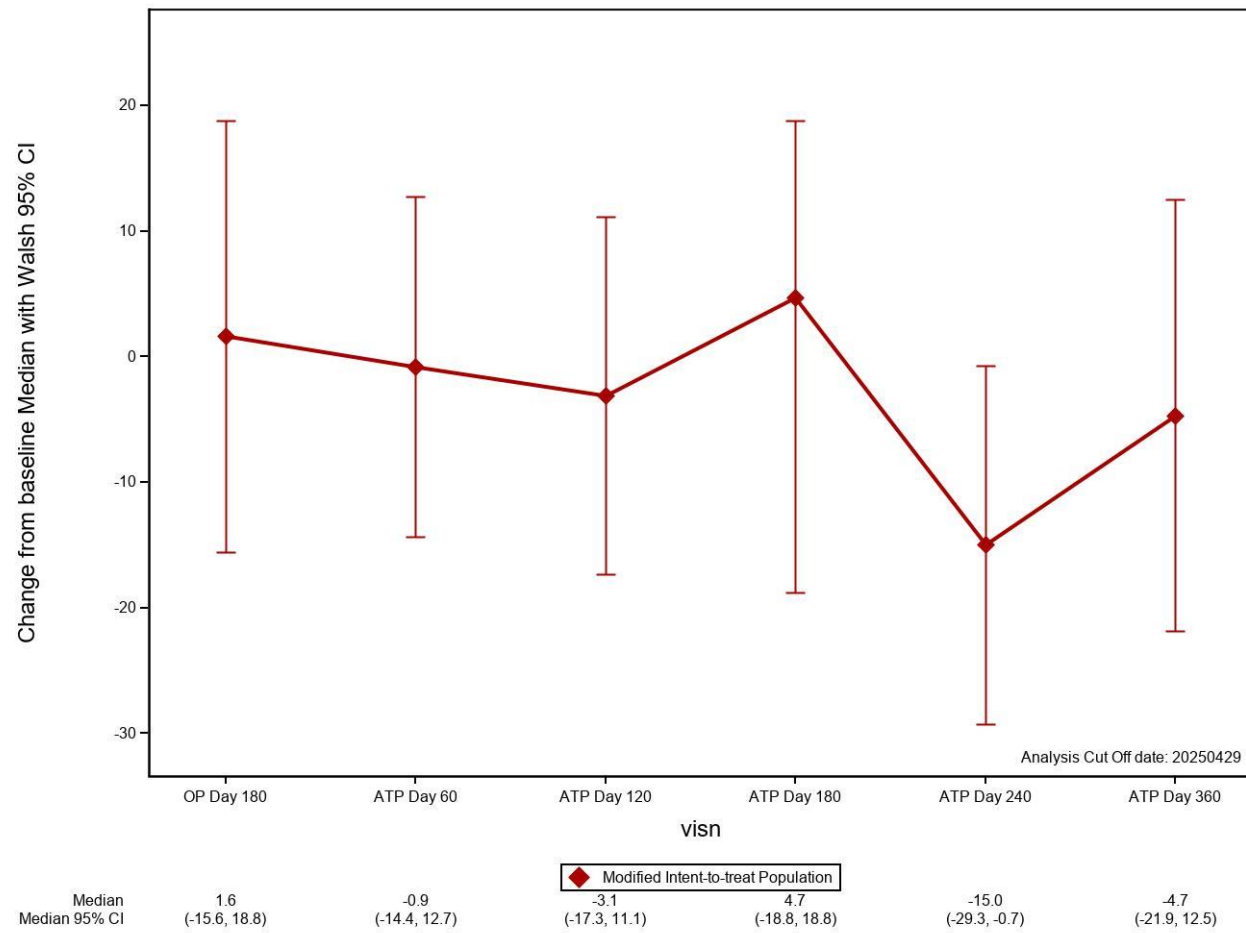
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.19.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Other People - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



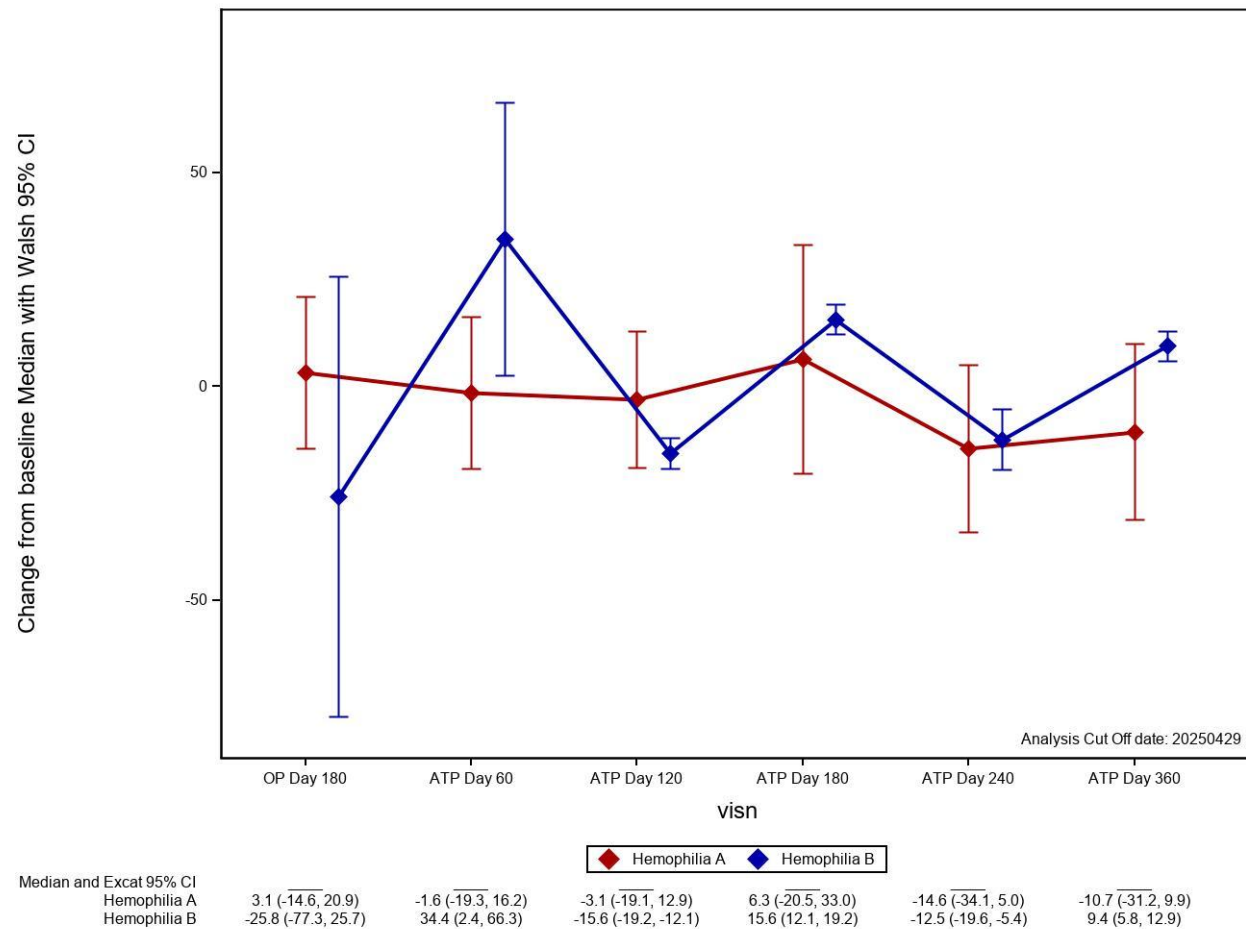
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.20.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Perceived Support - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



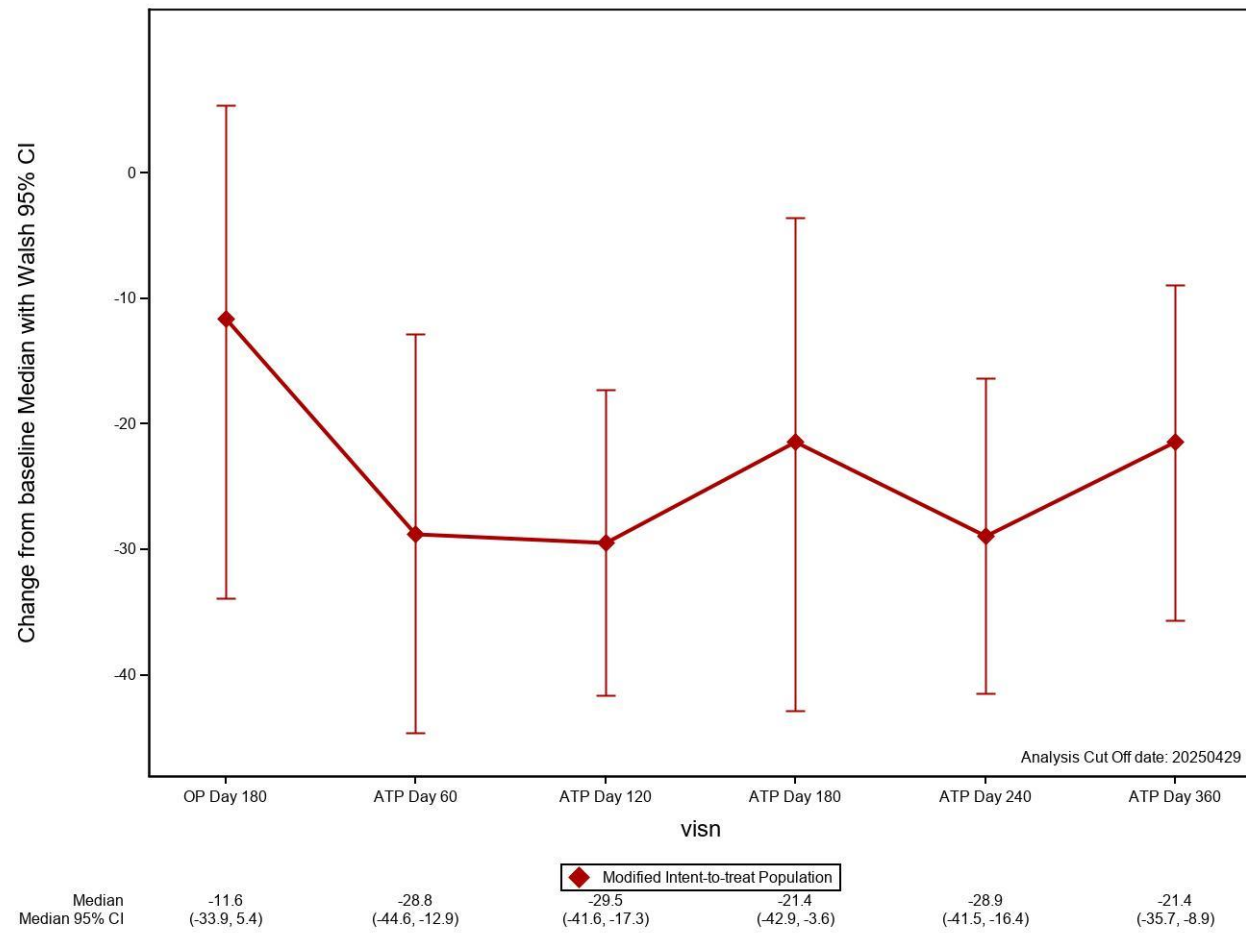
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.20.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Perceived Support - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



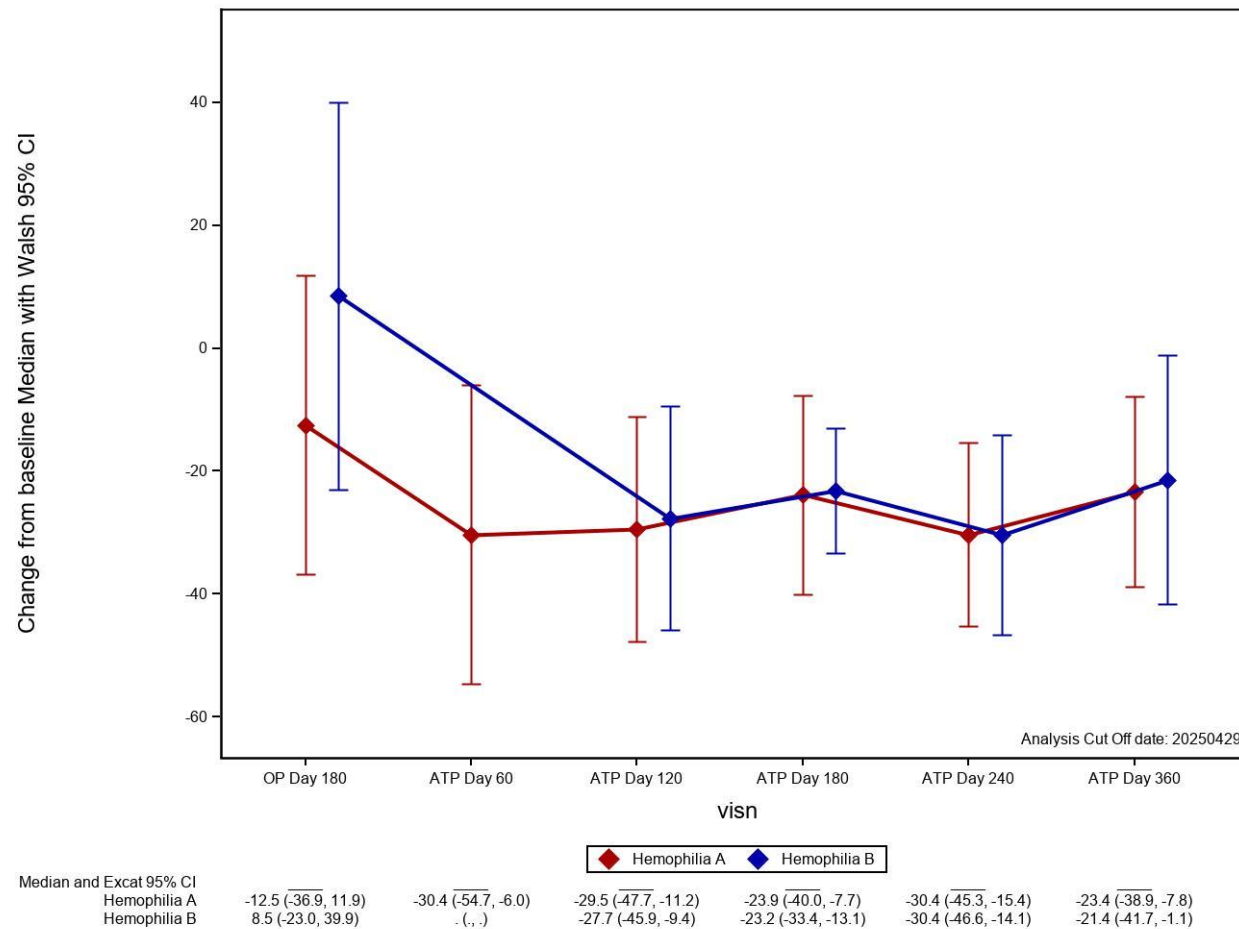
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.21.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Physical Health - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



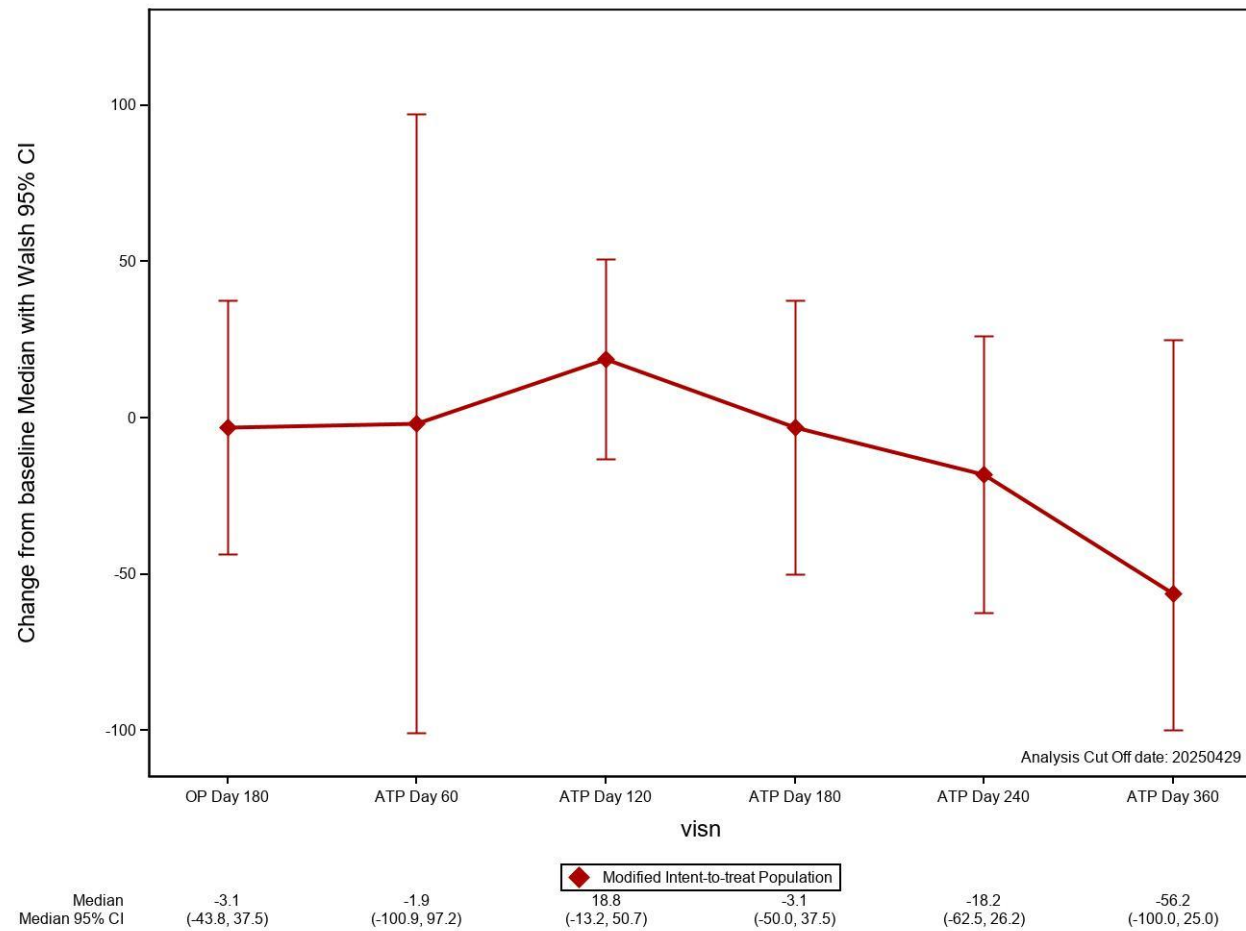
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.21.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Physical Health - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



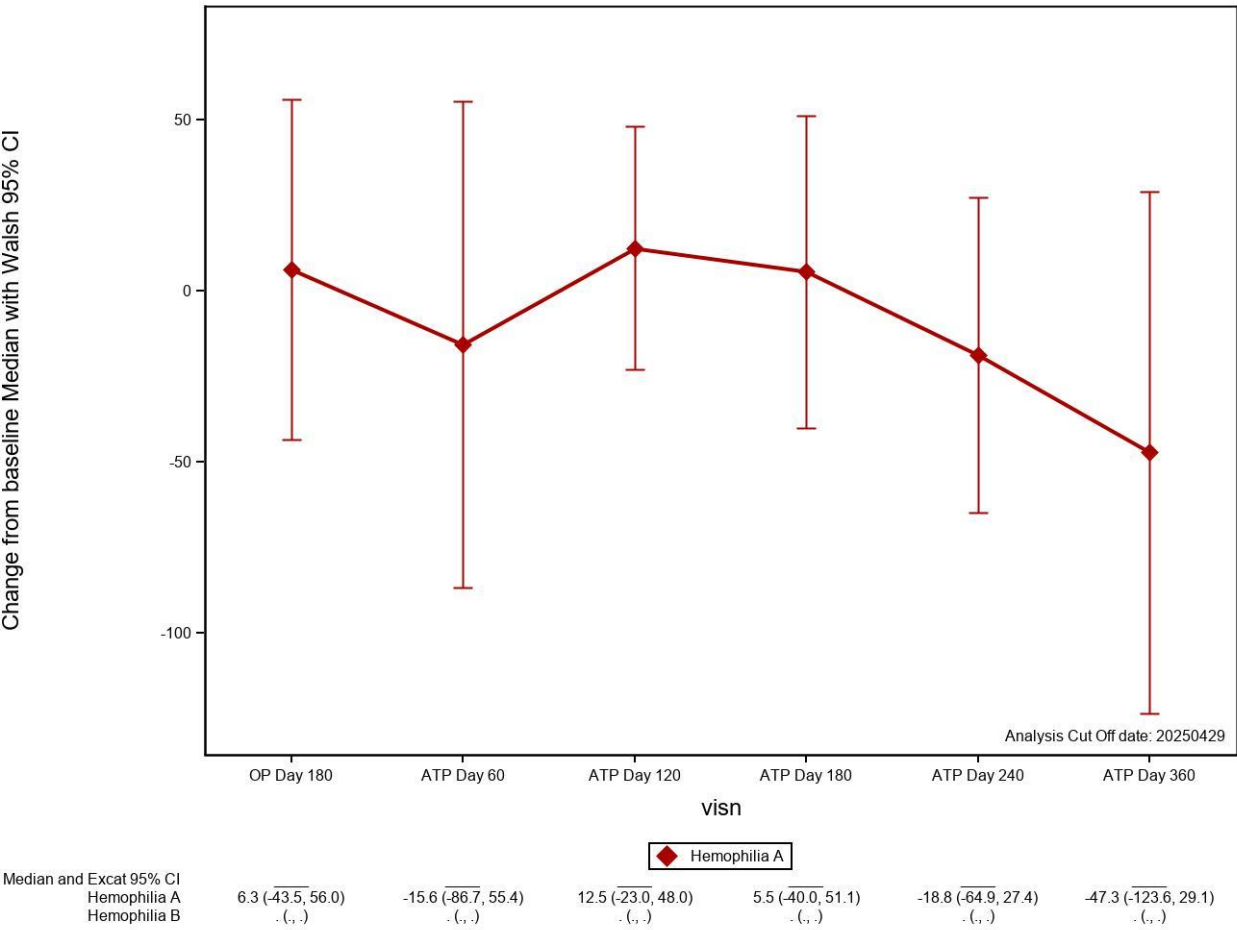
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.22.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Relationship - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



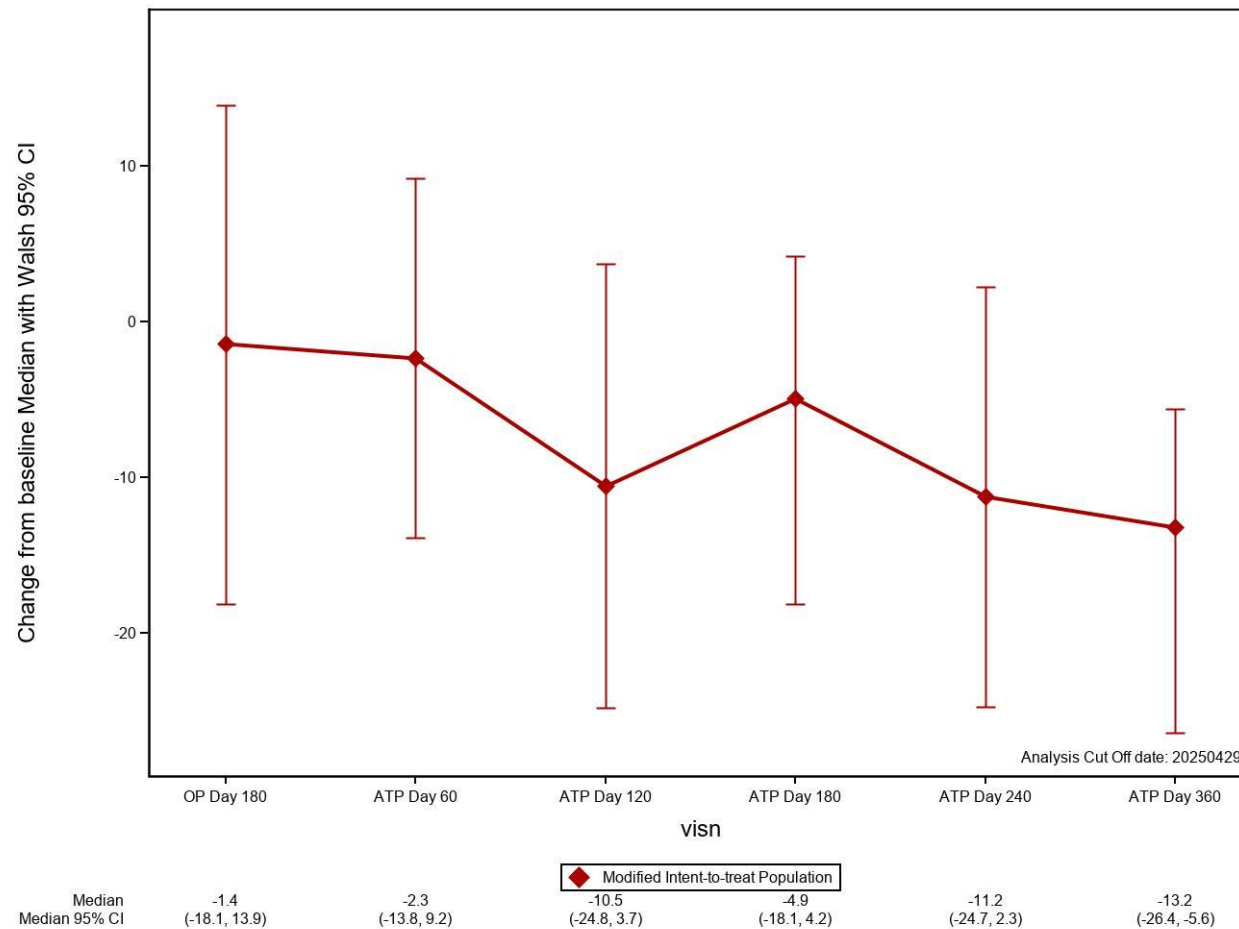
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.22.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Relationship - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



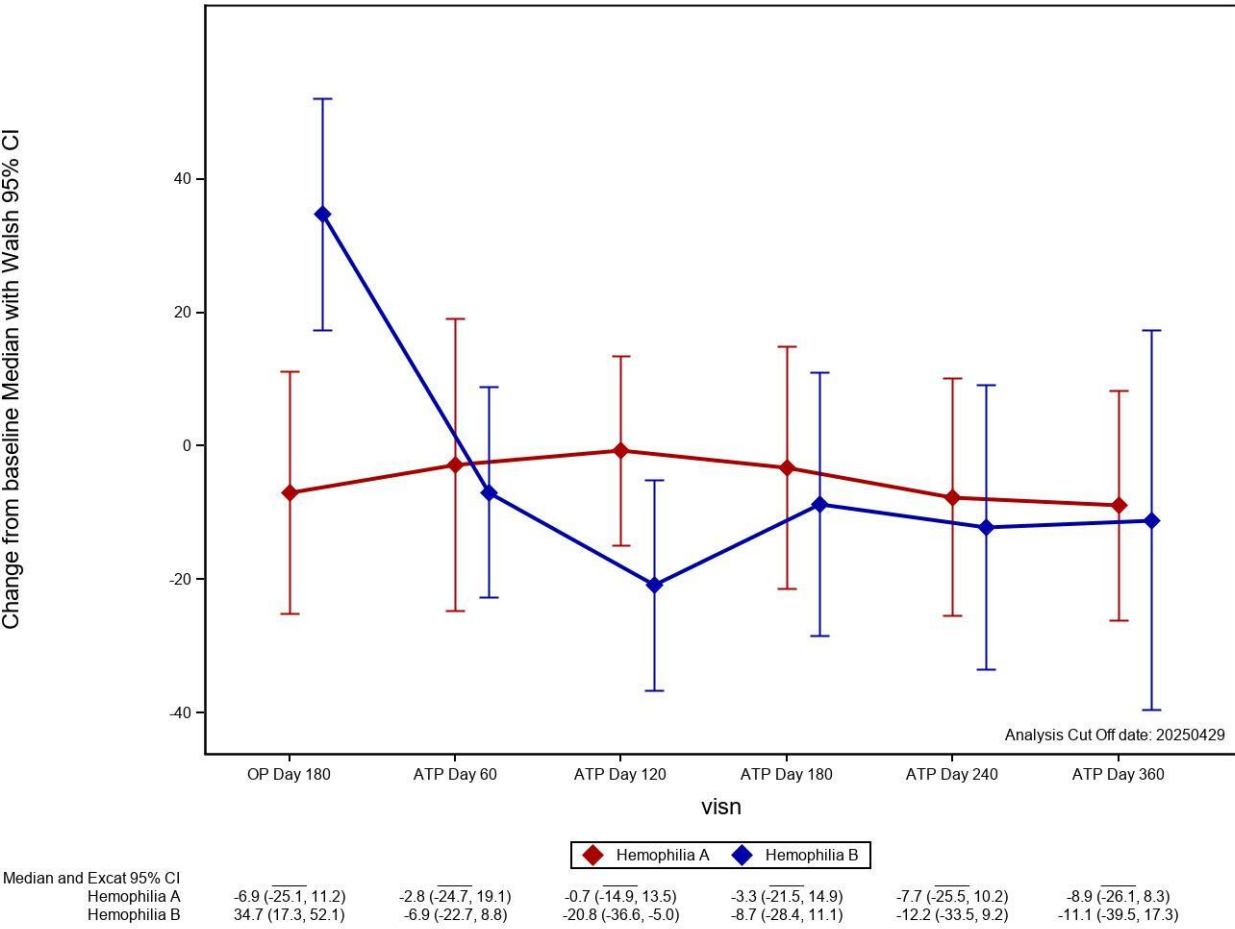
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.23.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Sports and School - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



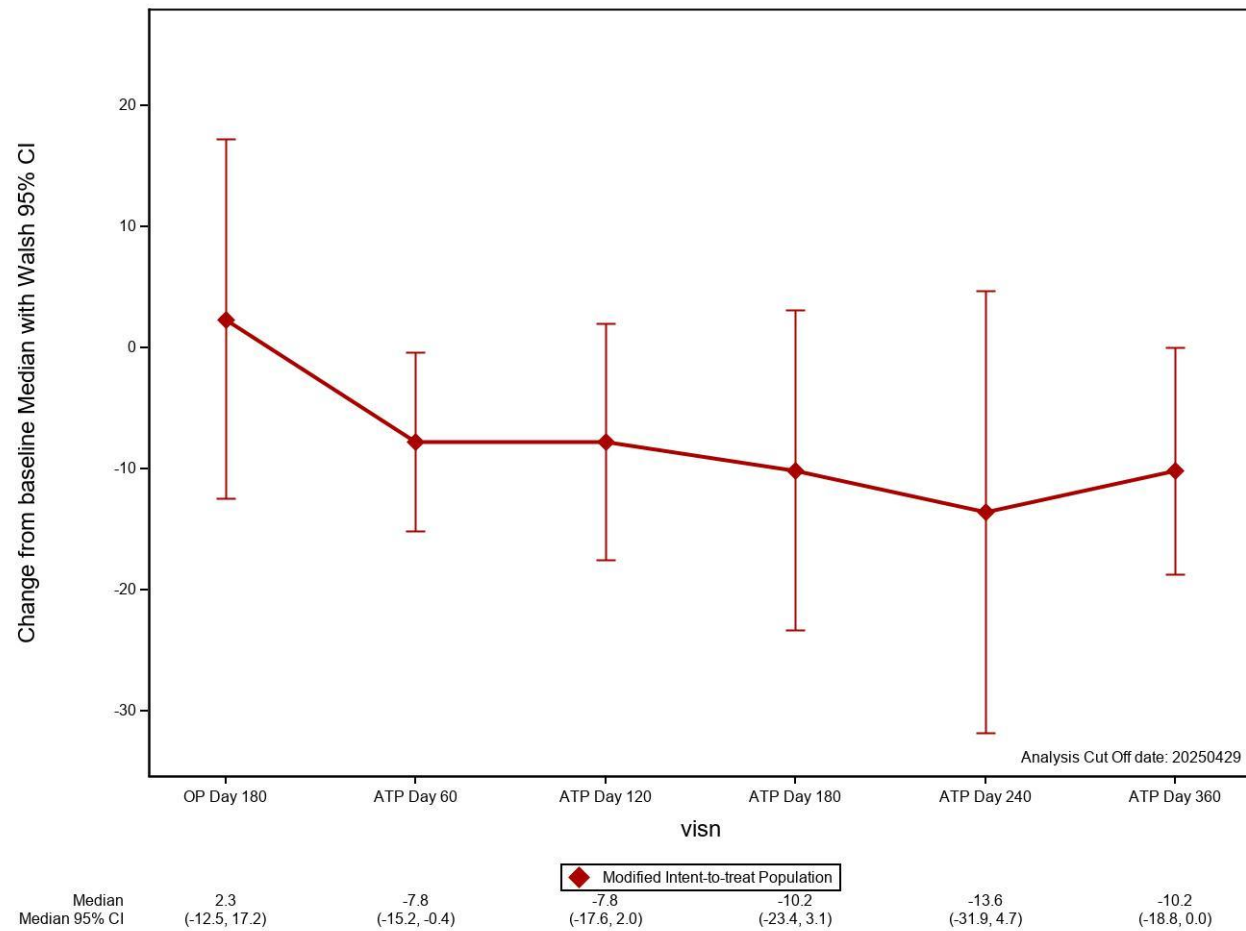
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.23.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Sports and School - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



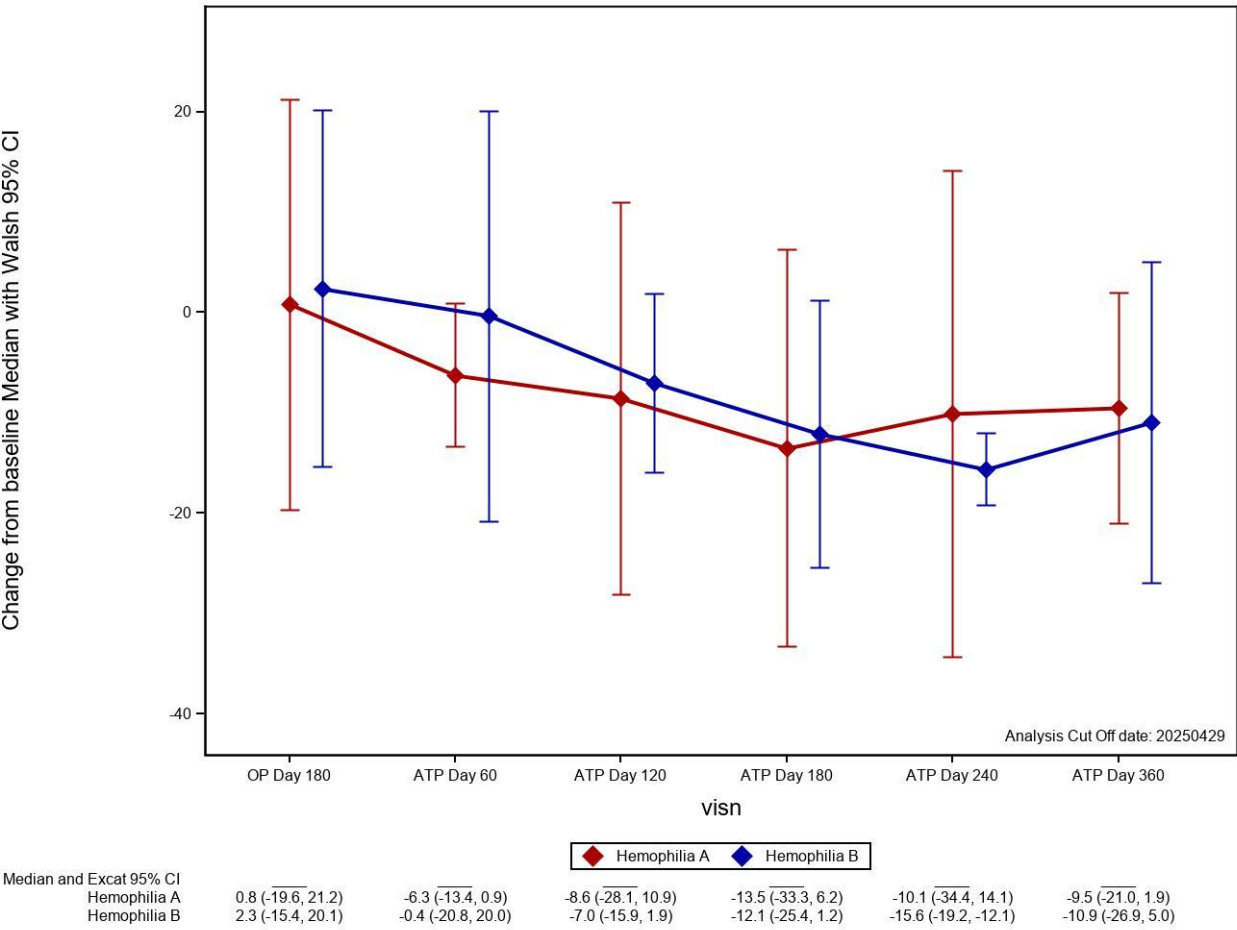
Medizinischer Nutzen, medizinischer Zusatznutzen, Patientengruppen mit therap. bedeutsamem Zusatznutzen

FIGURE .6.2.24.1: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Treatment - Modified Intent-to-Treat Set - Change from baseline Medians - Overall



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

FIGURE .6.2.24.2: Hemophilia Quality of Life Questionnaire for Children (Haem-QoL) - Treatment - Modified Intent-to-Treat Set - Change from baseline Medians - By Disease Type



9 Verträglichkeit

9.1 UE

9.1.1 Hauptanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.1. Incidence of Adverse event
 Safety Set

Table page 2 of 7.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]	
Adverse Event with exclusion of SOC/PTs related to underlying disease progression									
Overall									
Safety Set	48	16 (33.3%)	30 (62.5%)	35 (72.9%)	1.87 (1.21, 2.91) 0.0049	3.33 (1.50, 7.41) 0.0031	29.2 (10.9, 47.4) 0.0017		
Disease type									
Hemophilia A	40	12 (30.0%)	24 (60.0%)	29 (72.5%)	2.00 (1.19, 3.35) 0.0085	3.50 (1.45, 8.47) 0.0055	30.0 (10.2, 49.8) 0.0030		
Hemophilia B	8	4 (50.0%)	6 (75.0%)	6 (75.0%)	1.50 (0.67, 3.34) 0.3206	3.00 (0.36, 24.92) 0.3091	25.0 (-20.8, 70.8) 0.2850	0.8963	
Age group									
Adults (Ages 18 to < 75)	36	10 (27.8%)	23 (63.9%)	26 (72.2%)	2.30 (1.27, 4.16) 0.0058	4.60 (1.66, 12.78) 0.0034	36.1 (14.1, 58.1) 0.0013		
Adolescents (Ages 12 to < 18)	12	6 (50.0%)	7 (58.3%)	9 (75.0%)	1.17 (0.69, 1.97) 0.5641	1.40 (0.45, 4.34) 0.5600	8.3 (-19.6, 36.2) 0.5582	0.1468	
Region									
Asia	24	9 (37.5%)	20 (83.3%)	21 (87.5%)	-	-	-		
Europe	8	2 (25.0%)	5 (62.5%)	6 (75.0%)	-	-	-		
Other	16	5 (31.3%)	5 (31.3%)	8 (50.0%)	-	-	-		-
White Race									
White	15	4 (26.7%)	8 (53.3%)	10 (66.7%)	2.00 (0.75, 5.33) 0.1657	3.14 (0.66, 14.95) 0.1501	26.7 (-7.7, 61.1) 0.1287		
Other	33	12 (36.4%)	22 (66.7%)	25 (75.8%)	1.83 (1.13, 2.97) 0.0138	3.50 (1.36, 8.99) 0.0092	30.3 (8.9, 51.7) 0.0055	0.9083	

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

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

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.1.2 Sensitivitätsanalyse

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
Datacut: B7841005 (29APR2025)
TABLE 3.1. Incidence of Adverse event.
Safety Set

Table page 1 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP	Marstacimab Prophylaxis During ATP					OR
Event	N	n (%)	6 months n (%)	12 months n (%)	RR (95%CI) p-value [1]		OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	Interacti on p- value [2]
Adverse Event									
Overall									
Safety Set	48	16 (33.3%)	31 (64.6%)	35 (72.9%)	1.94 (1.27, 2.95) 0.0021		3.65 (1.68, 7.92) 0.0011	31.2 (13.8, 48.7) 0.0005	
Disease type									
Hemophilia A	40	12 (30.0%)	25 (62.5%)	29 (72.5%)	2.08 (1.27, 3.41) 0.0035		3.89 (1.66, 9.09) 0.0017	32.5 (13.7, 51.3) 0.0007	
Hemophilia B	8	4 (50.0%)	6 (75.0%)	6 (75.0%)	1.50 (0.67, 3.34) 0.3206		3.00 (0.36, 24.92) 0.3091	25.0 (-20.8, 70.8) 0.2850	0.8268
Age group									
Adults (Ages 18 to < 75)	36	10 (27.8%)	24 (66.7%)	26 (72.2%)	2.40 (1.36, 4.23) 0.0024		5.20 (1.94, 13.91) 0.0010	38.9 (18.1, 59.7) 0.0002	
Adolescents (Ages 12 to < 18)	12	6 (50.0%)	7 (58.3%)	9 (75.0%)	1.17 (0.69, 1.97) 0.5641		1.40 (0.45, 4.34) 0.5600	8.3 (-19.6, 36.2) 0.5582	0.1093
Region									
Asia	24	9 (37.5%)	20 (83.3%)	21 (87.5%)	-		-	-	
Europe	8	2 (25.0%)	5 (62.5%)	6 (75.0%)	-		-	-	
Other	16	5 (31.3%)	6 (37.5%)	8 (50.0%)	-		-	-	-
White Race									
White	15	4 (26.7%)	8 (53.3%)	10 (66.7%)	2.00 (0.75, 5.33) 0.1657		3.14 (0.66, 14.95) 0.1501	26.7 (-7.7, 61.1) 0.1287	
Other	33	12 (36.4%)	23 (69.7%)	25 (75.8%)	1.92 (1.21, 3.03) 0.0053		4.03 (1.63, 9.94) 0.0025	33.3 (13.3, 53.3) 0.0011	0.7904

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.1.3 UE nach SOC und PT

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.1. Incidence of Adverse event
 Safety Set

Table page 3 of 7.

Incidences by phase					ATP 6-months vs. OP 6-months		
		Inhibitor with On-Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months			
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]
SOC Gastrointestinal disorders							
-							
SOC overall	48	3 (6.3%)	3 (6.3%)	8 (16.7%)	1.00 (0.20, 4.95) 1.0000	1.00 (0.18, 5.51) 1.0000	-0.0 (-10.0, 10.0) 1.0000

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.1. Incidence of Adverse event
 Safety Set

Table page 4 of 7.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months		
		Inhibitor with On-Demand at OP	Marstacimab Prophylaxis	Marstacimab Prophylaxis	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]
			During ATP 6 months	During ATP 12 months			
SOC General disorders and administration site conditions							
-							
SOC overall	48	1 (2.1%)	7 (14.6%)	8 (16.7%)	7.00 (0.86, 56.89) 0.0687	8.02 (0.91, 71.06) 0.0613	12.5 (1.5, 23.5) 0.0259

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.1. Incidence of Adverse event
 Safety Set

Table page 5 of 7.

		Incidences by phase			ATP 6-months vs. OP 6-months		
		Inhibitor with On-Demand at OP	Marstacimab Prophylaxis During ATP 6 months	Marstacimab Prophylaxis During ATP 12 months			
Event	N	n (%)	n (%)	n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]
SOC Infections and infestations							
-							
SOC overall	48	5 (10.4%)	18 (37.5%)	25 (52.1%)	3.60 (1.46, 8.86) 0.0053	5.16 (1.74, 15.29) 0.0031	27.1 (11.0, 43.1) 0.0010
PT COVID-19	48	0 (0.0%)	8 (16.7%)	11 (22.9%)	9.00 (1.14, 71.04) 0.0371	10.85 (1.26, 93.70) 0.0303	16.7 (4.6, 28.7) 0.0066
PT Upper respiratory tract infection	48	0 (0.0%)	7 (14.6%)	8 (16.7%)	8.00 (1.00, 63.96) 0.0499	9.40 (1.08, 82.08) 0.0427	14.6 (3.0, 26.1) 0.0132

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.1. Incidence of Adverse event
 Safety Set

Table page 6 of 7.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months		
		Inhibitor with On-Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]
SOC Investigations							
-							
SOC overall	48	5 (10.4%)	8 (16.7%)	11 (22.9%)	1.60 (0.70, 3.63) 0.2612	1.72 (0.67, 4.39) 0.2562	6.2 (-4.4, 16.9) 0.2504

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

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

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.1. Incidence of Adverse event
 Safety Set

Table page 7 of 7.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months		
		Inhibitor with	Marstacimab	Marstacimab	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]
		On-Demand at OP n (%)	Prophylaxis During ATP 6 months n (%)	Prophylaxis During ATP 12 months n (%)			
SOC Nervous system disorders							
-							
SOC overall	48	2 (4.2%)	6 (12.5%)	6 (12.5%)	3.00 (0.61, 14.86) 0.1785	3.29 (0.59, 18.18) 0.1729	8.3 (-3.0, 19.6) 0.1486
PT Headache	48	1 (2.1%)	5 (10.4%)	5 (10.4%)	5.00 (0.58, 42.80) 0.1418	5.47 (0.59, 50.72) 0.1351	8.3 (-1.4, 18.1) 0.0929

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.2 SUE

9.2.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 3.2. Incidence of Serious adverse event

Safety Set

Table page 2 of 2.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]	
Serious Adverse Event with exclusion of SOCs/PTs related to underlying disease progression									
Overall									
Safety Set	48	3 (6.3%)	0 (0.0%)	1 (2.1%)	0.25 (0.03, 2.24) 0.2150	0.23 (0.02, 2.26) 0.2097	-6.3 (-15.2, 2.7) 0.1715		
Disease type									
Hemophilia A	40	1 (2.5%)	0 (0.0%)	0 (0.0%)	0.50 (0.05, 5.51) 0.5714	0.49 (0.04, 5.84) 0.5704	-2.5 (-11.0, 6.0) 0.5621		
Hemophilia B	8	2 (25.0%)	0 (0.0%)	1 (12.5%)	0.33 (0.03, 3.20) 0.3414	0.24 (0.01, 4.17) 0.3258	-25.0 (-70.8, 20.8) 0.2850	0.7146	
Age group									
Adults (Ages 18 to < 75)	36	1 (2.8%)	0 (0.0%)	1 (2.8%)	0.50 (0.05, 5.51) 0.5714	0.49 (0.04, 5.88) 0.5703	-2.8 (-12.2, 6.6) 0.5619		
Adolescents (Ages 12 to < 18)	12	2 (16.7%)	0 (0.0%)	0 (0.0%)	0.33 (0.03, 3.20) 0.3414	0.27 (0.02, 3.72) 0.3298	-16.7 (-47.9, 14.6) 0.2963	0.7569	
Region									
Asia	24	0 (0.0%)	0 (0.0%)	0 (0.0%)	-	-	-		
Europe	8	0 (0.0%)	0 (0.0%)	1 (12.5%)	-	-	-		
Other	16	3 (18.8%)	0 (0.0%)	0 (0.0%)	-	-	-		-
White Race									
White	15	1 (6.7%)	0 (0.0%)	1 (6.7%)	0.50 (0.05, 5.51) 0.5714	0.46 (0.03, 6.50) 0.5688	-6.7 (-29.0, 15.7) 0.5593		
Other	33	2 (6.1%)	0 (0.0%)	0 (0.0%)	0.33 (0.03, 3.20) 0.3414	0.31 (0.03, 3.36) 0.3368	-6.1 (-17.8, 5.6) 0.3099	0.8282	

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

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

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.2.2 Sensitivitätsanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.2. Incidence of Serious adverse event.
 Safety Set

Table page 1 of 2.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP	Marstacimab Prophylaxis During ATP					OR Interacti
Event	N	n (%)	6 months n (%)	12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		on p- value [2]
Serious Adverse Event									
Overall									
Safety Set	48	3 (6.3%)	0 (0.0%)	1 (2.1%)	0.25 (0.03, 2.24) 0.2150	0.23 (0.02, 2.26) 0.2097	-6.3 (-15.2, 2.7) 0.1715		
Disease type									
Hemophilia A	40	1 (2.5%)	0 (0.0%)	0 (0.0%)	0.50 (0.05, 5.51) 0.5714	0.49 (0.04, 5.84) 0.5704	-2.5 (-11.0, 6.0) 0.5621		
Hemophilia B	8	2 (25.0%)	0 (0.0%)	1 (12.5%)	0.33 (0.03, 3.20) 0.3414	0.24 (0.01, 4.17) 0.3258	-25.0 (-70.8, 20.8) 0.2850	0.7146	
Age group									
Adults (Ages 18 to < 75)	36	1 (2.8%)	0 (0.0%)	1 (2.8%)	0.50 (0.05, 5.51) 0.5714	0.49 (0.04, 5.88) 0.5703	-2.8 (-12.2, 6.6) 0.5619		
Adolescents (Ages 12 to < 18)	12	2 (16.7%)	0 (0.0%)	0 (0.0%)	0.33 (0.03, 3.20) 0.3414	0.27 (0.02, 3.72) 0.3298	-16.7 (-47.9, 14.6) 0.2963	0.7569	
Region									
Asia	24	0 (0.0%)	0 (0.0%)	0 (0.0%)	-	-	-		
Europe	8	0 (0.0%)	0 (0.0%)	1 (12.5%)	-	-	-		
Other	16	3 (18.8%)	0 (0.0%)	0 (0.0%)	-	-	-		-
White Race									
White	15	1 (6.7%)	0 (0.0%)	1 (6.7%)	0.50 (0.05, 5.51) 0.5714	0.46 (0.03, 6.50) 0.5688	-6.7 (-29.0, 15.7) 0.5593		
Other	33	2 (6.1%)	0 (0.0%)	0 (0.0%)	0.33 (0.03, 3.20) 0.3414	0.31 (0.03, 3.36) 0.3368	-6.1 (-17.8, 5.6) 0.3099	0.8282	

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.3 Schwere UE

9.3.1 Hauptanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.3. Incidence of Severe adverse event
 Safety Set

Table page 2 of 2.

Event	N	Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab	Marstacimab	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]	OR Interacti on p- value [2]	
			Prophylaxis During ATP 6 months n (%)	Prophylaxis During ATP 12 months n (%)					
Severe Adverse Event with exclusion of SOC/PTs related to underlying disease progression									
Overall									
Safety Set	48	3 (6.3%)	2 (4.2%)	3 (6.3%)	0.67 (0.11, 3.99) 0.6569	0.65 (0.10, 4.29) 0.6565	-2.1 (-11.2, 7.0) 0.6540		
Disease type									
Hemophilia A	40	1 (2.5%)	2 (5.0%)	2 (5.0%)	2.00 (0.18, 22.06) 0.5714	2.05 (0.17, 24.60) 0.5704	2.5 (-6.0, 11.0) 0.5621		
Hemophilia B	8	2 (25.0%)	0 (0.0%)	1 (12.5%)	0.33 (0.03, 3.20) 0.3414	0.24 (0.01, 4.17) 0.3258	-25.0 (-70.8, 20.8) 0.2850	0.2603	
Age group									
Adults (Ages 18 to < 75)	36	1 (2.8%)	2 (5.6%)	3 (8.3%)	2.00 (0.18, 22.06) 0.5714	2.06 (0.17, 24.92) 0.5703	2.8 (-6.6, 12.2) 0.5619		
Adolescents (Ages 12 to < 18)	12	2 (16.7%)	0 (0.0%)	0 (0.0%)	0.33 (0.03, 3.20) 0.3414	0.27 (0.02, 3.72) 0.3298	-16.7 (-47.9, 14.6) 0.2963	0.2674	
Region									
Asia	24	0 (0.0%)	2 (8.3%)	2 (8.3%)	-	-	-		
Europe	8	0 (0.0%)	0 (0.0%)	1 (12.5%)	-	-	-		
Other	16	3 (18.8%)	0 (0.0%)	0 (0.0%)	-	-	-	-	
White Race									
White	15	1 (6.7%)	0 (0.0%)	1 (6.7%)	0.50 (0.05, 5.51) 0.5714	0.46 (0.03, 6.50) 0.5688	-6.7 (-29.0, 15.7) 0.5593		
Other	33	2 (6.1%)	2 (6.1%)	2 (6.1%)	1.00 (0.14, 7.10) 1.0000	1.00 (0.12, 8.06) 1.0000	-0.0 (-11.9, 11.9) 1.0000	0.6515	

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

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

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.3.2 Sensitivitätsanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.3. Incidence of Severe adverse event.
 Safety Set

Table page 1 of 2.

		Incidences by phase			ATP 6-months vs. OP 6-months				
		Inhibitor with On- Demand at OP	Marstacimab Prophylaxis During ATP	Marstacimab Prophylaxis During ATP					OR Interacti
Event	N	n (%)	6 months n (%)	12 months n (%)	RR (95%CI) p-value [1]	OR (95%CI) p-value [2]	RD (95%CI) p-value [3]		on p- value [2]
Severe Adverse Event									
Overall									
Safety Set	48	3 (6.3%)	3 (6.3%)	4 (8.3%)	1.00 (0.20, 4.95) 1.0000	1.00 (0.18, 5.51) 1.0000	-0.0 (-10.0, 10.0) 1.0000		
Disease type									
Hemophilia A	40	1 (2.5%)	2 (5.0%)	2 (5.0%)	2.00 (0.18, 22.06) 0.5714	2.05 (0.17, 24.60) 0.5704	2.5 (-6.0, 11.0) 0.5621		
Hemophilia B	8	2 (25.0%)	1 (12.5%)	2 (25.0%)	0.50 (0.05, 5.51) 0.5714	0.43 (0.02, 7.80) 0.5670	-12.5 (-54.0, 29.0) 0.5553	0.4159	
Age group									
Adults (Ages 18 to < 75)	36	1 (2.8%)	3 (8.3%)	4 (11.1%)	3.00 (0.31, 28.84) 0.3414	3.18 (0.30, 33.83) 0.3372	5.6 (-5.2, 16.3) 0.3105		
Adolescents (Ages 12 to < 18)	12	2 (16.7%)	0 (0.0%)	0 (0.0%)	0.33 (0.03, 3.20) 0.3414	0.27 (0.02, 3.72) 0.3298	-16.7 (-47.9, 14.6) 0.2963	0.1581	
Region									
Asia	24	0 (0.0%)	3 (12.5%)	3 (12.5%)	-	-	-		
Europe	8	0 (0.0%)	0 (0.0%)	1 (12.5%)	-	-	-		
Other	16	3 (18.8%)	0 (0.0%)	0 (0.0%)	-	-	-		-
White Race									
White	15	1 (6.7%)	0 (0.0%)	1 (6.7%)	0.50 (0.05, 5.51) 0.5714	0.46 (0.03, 6.50) 0.5688	-6.7 (-29.0, 15.7) 0.5593		
Other	33	2 (6.1%)	3 (9.1%)	3 (9.1%)	1.50 (0.25, 8.98) 0.6569	1.55 (0.22, 10.68) 0.6563	3.0 (-10.2, 16.3) 0.6537	0.4652	

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

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

[1]RR: Risk Ratio: Poisson model (log link, robust SEs) to estimate risk ratios for ATP (6 months) vs OP (6 months).

[2]OR: Odds Ratio: Logistic regression model (logit link) to estimate odds ratios for ATP (6 months) vs OP (6 months).

[3]RD: Risk Difference: Binomial generalized linear model with identity link to estimate risk differences for ATP (6 months) vs OP (6 months).

9.4 UE, die zum Therapieabbruch führten

9.4.1 Hauptanalyse

Pfizer Inc.: Final

Marstacimab

Inhibitor with On-Demand at OP

Datacut: B7841005 (29APR2025)

TABLE 3.4. Incidence of Treatment discontinuation due to adverse event

Safety Set

Table page 2 of 3.

Event	N	Incidences by phase		
		Inhibitor with On-Demand at OP n (%)	Marstacimab Prophylaxis	Marstacimab Prophylaxis
			During ATP 6 months n (%)	During ATP 12 months n (%)
Treatment Discontinuation Due To Adverse Event with exclusion of SOCs/PTs related to underlying disease progression				
Overall				
Safety Set	48	0 (0.0%)	0 (0.0%)	1 (2.1%)
Disease type				
Hemophilia A	40	0 (0.0%)	0 (0.0%)	0 (0.0%)
Hemophilia B	8	0 (0.0%)	0 (0.0%)	1 (12.5%)
Age group				
Adults (Ages 18 to < 75)	36	0 (0.0%)	0 (0.0%)	1 (2.8%)
Adolescents (Ages 12 to < 18)	12	0 (0.0%)	0 (0.0%)	0 (0.0%)
Region				
Asia	24	0 (0.0%)	0 (0.0%)	0 (0.0%)
Europe	8	0 (0.0%)	0 (0.0%)	1 (12.5%)
Other	16	0 (0.0%)	0 (0.0%)	0 (0.0%)
White Race				
White	15	0 (0.0%)	0 (0.0%)	1 (6.7%)
Other	33	0 (0.0%)	0 (0.0%)	0 (0.0%)

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

9.4.2 Sensitivitätsanalyse

Pfizer Inc.: Final
 Marstacimab
 Inhibitor with On-Demand at OP
 Datacut: B7841005 (29APR2025)
 TABLE 3.4. Incidence of Treatment discontinuation due to adverse event.
 Safety Set

Table page 1 of 3.

Event	N	Incidences by phase		
		Inhibitor with On-Demand at OP n (%)	Marstacimab Prophylaxis During ATP 6 months n (%)	Marstacimab Prophylaxis During ATP 12 months n (%)
Treatment Discontinuation Due To Adverse Event				
Overall				
Safety Set	48	0 (0.0%)	0 (0.0%)	1 (2.1%)
Disease type				
Hemophilia A	40	0 (0.0%)	0 (0.0%)	0 (0.0%)
Hemophilia B	8	0 (0.0%)	0 (0.0%)	1 (12.5%)
Age group				
Adults (Ages 18 to < 75)	36	0 (0.0%)	0 (0.0%)	1 (2.8%)
Adolescents (Ages 12 to < 18)	12	0 (0.0%)	0 (0.0%)	0 (0.0%)
Region				
Asia	24	0 (0.0%)	0 (0.0%)	0 (0.0%)
Europe	8	0 (0.0%)	0 (0.0%)	1 (12.5%)
Other	16	0 (0.0%)	0 (0.0%)	0 (0.0%)
White Race				
White	15	0 (0.0%)	0 (0.0%)	1 (6.7%)
Other	33	0 (0.0%)	0 (0.0%)	0 (0.0%)

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.

AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.

In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.

Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.

ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.

9.4.3 UE, die zum Therapieabbruch führten nach SOC und PT

Pfizer Inc.: Final
Marstacimab
Inhibitor with On-Demand at OP
Datacut: B7841005 (29APR2025)
TABLE 3.4. Incidence of Treatment discontinuation due to adverse event
Safety Set

Table page 3 of 3.

Event	N	Incidences by phase		
		Inhibitor with On-Demand at OP n (%)	Marstacimab Prophylaxis	Marstacimab Prophylaxis
			During ATP	During ATP
			6 months n (%)	12 months n (%)
SOC Skin and subcutaneous tissue disorders				
-				
SOC overall	48	0 (0.0%)	0 (0.0%)	1 (2.1%)
PT Rash	48	0 (0.0%)	0 (0.0%)	1 (2.1%)

Inhibitor Marstacimab safety set is defined as those patients who took at least 1 dose of Marstacimab.
AEs with onset from Visit 2 up to, but prior to, the first dose of Marstacimab in the ATP were attributed to OP. AEs with onset on or after the first dose of Marstacimab in ATP up to the last study contact day were attributed to ATP. AE data reported for the ATP 12-month period are cumulative and also include all events occurring during the ATP 6-month period.
In the case of zero events in either the OP or the ATP-6-months phase, a correction is applied: 1 subject without any event (in OP and ATP-6-months) is assigned an event in the OP, and another subject without any event is assigned an event in the ATP-6-month period.
Subgroup analyses were not performed for cases where at least 1 subgroup had N subjects < 10.
ATP: Active Treatment Phase; CI: Confidence Interval; N: number of patients in the All-Safety-Set; n: number of patients with an event; NE: not estimable; OP: Observational Phase.