Inhaltsverzeichnis

POPULATION: Intent-to-Treat Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Demographics and Baseline Characteristics

	Patients with 2 SMN2 copies (N=8)	Patients with 3 SMN2 copies (N=13)	Patients with >=4 SMN2 copies (N=5)	All Patients (N=26)
Age at Enrollment (Days)				
n	8	13	5	26
Mean (SD)	22.75 (4.98)	28.92 (7.52)	31.20 (6.06)	27.46 (7.14)
Median	22.5	27	31	24
IQR	21.0 - 23.5	23.0 - 36.0	28.0 - 36.0	22.0 - 36.0
Min - Max	15.0 - 33.0	19.0 - 40.0	23.0 - 38.0	15.0 - 40.0
Age at First Dose (Days)				
n	8	13	5	26
Mean (SD)	23.9 (5.3)	29.9 (7.5)	32.4 (6.3)	28.5 (7.2)
Median	23.5	28	32	25
25% and 75%-ile	22 - 25	24 - 37	29 - 37	23 - 37
Min - Max	16 - 35	20 - 41	24 - 40	16 - 41
Sex				
n	8	13	5	26
Male	4 (50.0%)	4 (30.8%)	2 (40.0%)	10 (38.5%)
Female	4 (50.0%)	9 (69.2%)	3 (60.0%)	16 (61.5%)
Race				
n	8	13	5	26
Asian	0	1 (7.7%)	2 (40.0%)	3 (11.5%)
White	8 (100%)	11 (84.6%)	3 (60.0%)	22 (84.6%)
Unknown	0	1 (7.7%)	0	1 (3.8%)

Ethnicity				
n	8	13	5	26
Hispanic or Latino	3 (37.5%)	0	0	3 (11.5%)
Not Hispanic or Latino	5 (62.5%)	12 (92.3%)	5 (100%)	22 (84.6%)
Not Stated	0	1 (7.7%)	0	1 (3.8%)
Country				
n	8	13	5	26
Australia	2 (25.0%)	6 (46.2%)	0	8 (30.8%)
Belgium	0	1 (7.7%)	2 (40.0%)	3 (11.5%)
Brazil	3 (37.5%)	0	0	3 (11.5%)
POL	1 (12.5%)	1 (7.7%)	1 (20.0%)	3 (11.5%)
Russian Federation	2 (25.0%)	3 (23.1%)	0	5 (19.2%)
Taiwan	0	0	2 (40.0%)	2 (7.7%)
United States	0	2 (15.4%)	0	2 (7.7%)
Region				
n	8	13	5	26
Europe	3 (37.5%)	5 (38.5%)	3 (60.0%)	11 (42.3%)
Rest of the World	5 (62.5%)	6 (46.2%)	2 (40.0%)	13 (50.0%)
US	0	2 (15.4%)	0	2 (7.7%)
Baseline Weight (g)				
n	8	13	5	26
Mean (SD)	3820.5 (435.1)	4060.1 (647.5)	4190.0 (902.9)	4011.3 (635.6)
Median	3999	4000	4170	4015
25% and 75%-ile	3538 - 4073	3560 - 4345	3585 - 4300	3560 - 4270
Min - Max	3076 - 4270	3400 - 5726	3275 - 5620	3076 - 5726
Baseline Height/Length (cm)				
n	8	13	5	26
Mean (SD)	53.88 (3.72)	53.46 (2.73)	54.60 (2.70)	53.81 (2.97)
Median	53	53	53	53
25% and 75%-ile	52.5 - 54.5	51.0 - 55.0	53.0 - 57.0	52.0 - 55.0
Min - Max	49.0 - 62.0	50.0 - 59.0	52.0 - 58.0	49.0 - 62.0
SMA Identification Method				

n	8	13	5	26
Family History	4 (50.0%)	1 (7.7%)	0	5 (19.2%)
Newborn Screening	4 (50.0%)	11 (84.6%)	5 (100%)	20 (76.9%)
Other	0	1 (7.7%)	0	1 (3.8%)
CHOP-INTEND Score				
n	8	13	5	26
Mean (SD)	44.88 (6.56)	53.77 (5.76)	48.60 (3.44)	50.04 (6.79)
Median	46.5	55	50	51.5
IQR	39.5 - 50.0	52.0 - 57.0	46.0 - 51.0	46.0 - 55.0
Min - Max	35.0 - 52.0	44.0 - 62.0	44.0 - 52.0	35.0 - 62.0
HINE-2 Score				
n	8	13	5	26
Mean (SD)	2.13 (1.46)	3.00 (1.41)	2.00 (1.41)	2.54 (1.45)
Median	2	3	1	2.5
IQR	1.0 - 3.5	2.0 - 4.0	1.0 - 3.0	1.0 - 4.0
Min - Max	0.0 - 4.0	1.0 - 6.0	1.0 - 4.0	0.0 - 6.0
CMAP Amplitude Value (mV)				
n	8	13	5	26
Mean (SD)	1.97 (1.17)	4.42 (1.29)	4.38 (1.32)	3.66 (1.67)
Median	2.01	4.6	3.7	3.6
IQR	1.0 - 2.8	3.5 - 5.1	3.6 - 4.6	2.6 - 4.6
Min - Max	0.5 - 3.8	2.1 - 6.7	3.4 - 6.6	0.5 - 6.7
CMAP Amplitude Category				
n	8	13	5	26
< 1.5mV	3 (37.5%)	0	0	3 (11.5%)
>= 1.5mV	5 (62.5%)	13 (100%)	5 (100%)	23 (88.5%)

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_dm.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_dm_IT_20FEB2023_40703.xls
17AUG2023 10:28

POPULATION: Intent-to-Treat Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Patient Disposition

	Patients with 2 SMN2 copies (N=8)	Patients with 3 SMN2 copies (N=13)	Patients with >=4 SMN2 copies (N=5)	All Patients (N=26)
Enrolled	8 (100%)	13 (100%)	5 (100%)	26 (100%)
Ongoing	5 (62.5%)	13 (100%)	5 (100%)	23 (88.5%)
Completed 12 months of treatment	7 (87.5%)	13 (100%)	5 (100%)	25 (96.2%)
Completed 24 months of treatment	4 (50.0%)	5 (38.5%)	2 (40.0%)	11 (42.3%)
Discontinued between 0 and 12 months of treatment	1 (12.5%)	0	0	1 (3.8%)
Withdrawal by Subject	1 (12.5%)	0	0	1 (3.8%)
Discontinued between 12 and 24 months of treatment	2 (25.0%)	0	0	2 (7.7%)
Withdrawal by Subject	2 (25.0%)	0	0	2 (7.7%)
Entered extension phase	4 (50.0%)	4 (30.8%)	2 (40.0%)	10 (38.5%)
Discontinued from extension phase	0	0	0	0
Completed extension phase	0	0	0	0
Entered safety follow-up	3 (37.5%)	0	0	3 (11.5%)
Discontinued from safety follow-up	0	0	0	0
Completed safety follow-up	3 (37.5%)	0	0	3 (11.5%)

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ds.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ds_IT_20FEB2023_40703.xls
17AUG2023 10:27

POPULATION: Safety-Evaluable Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Duration of Follow-up for Safety

	Patients with 2 SMN2 copies (N=8)	Patients with 3 SMN2 copies (N=13)	Patients with >=4 SMN2 copies (N=5)	All Patients (N=26)
Follow-up Duration (days)				
n	8	13	5	26
Median	915	630	636	710

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_fu.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_fu_SE_20FEB2023_40703.xls
17AUG2023 10:30

POPULATION: Intent-to-Treat Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Duration of Follow-up for Efficacy

	Patients with 2 SMN2 copies (N=8)	Patients with 3 SMN2 copies (N=13)	Patients with >=4 SMN2 copies (N=5)	All Patients (N=26)
Follow-up Duration (days)				
n	8	13	5	26
Median	702	630	636	633

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_fu2.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_fu2_IT_20FEB2023_40703.xls
17AUG2023 10:29

POPULATION: Safety-Evaluable Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Number of Patients who Died including Primary Reason

Null Report: No observations met the reporting criteria for inclusion in this output.

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_dd.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_dd_SE_20FEB2023_40703.xls
17AUG2023 10:29

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death (from enrollment)
MODEL: Unstratified Analysis
STUDY: BN40703
Time to Event Analysis (Efficacy)

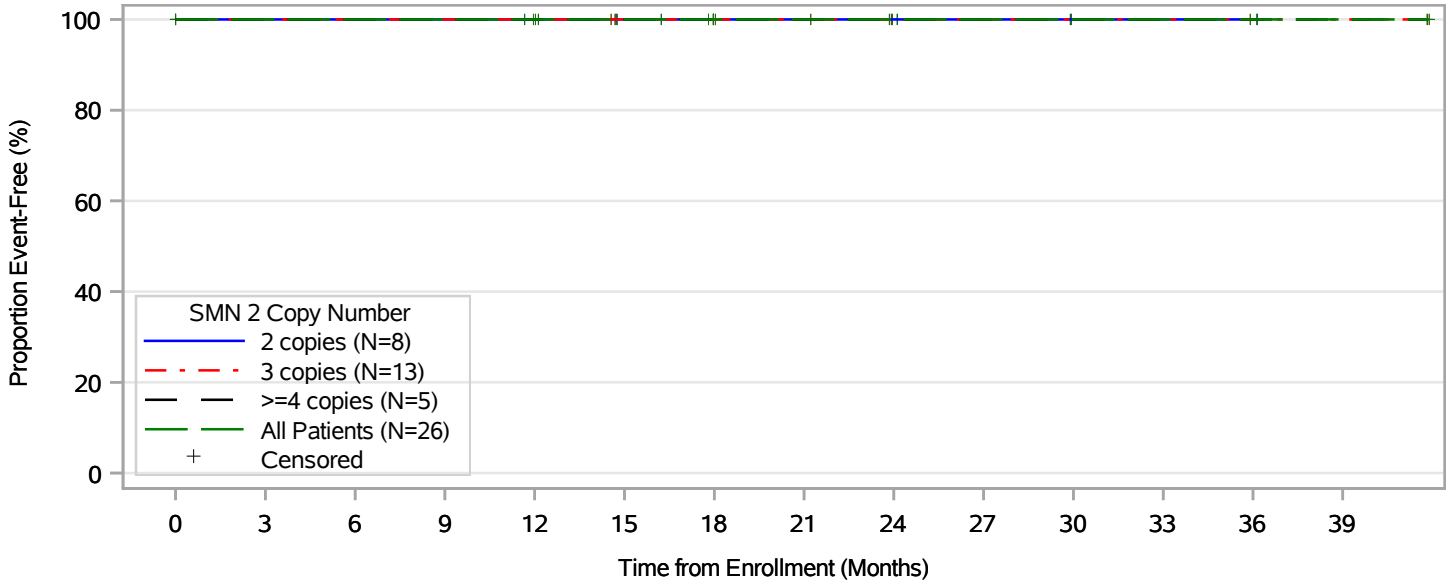
		Patients with 2 SMN2 copies (N=8)												Patients with 3 SMN2 copies (N=13)											
		Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
Name	Level	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
All	n/a	8	100.0	0	0	8	100.0	NE	NE	NE	NE	NE	NE	13	100.0	0	0	13	100.0	NE	NE	NE	NE	NE	NE

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_tte.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_tte_DTH_IT_20FEB2023_40703.xls
17AUG2023 12:03

Patients with >=4 SMN2 copies (N=5)												All Patients (N=26)											
Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
5	100.0	0	0	5	100.0	NE	NE	NE	NE	NE	NE	26	100.0	0	0	26	100.0	NE	NE	NE	NE	NE	NE

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death (from enrollment)
STUDY: BN40703



Patients at risk														
2 copies (N=8)	8	8	8	8	7	6	4	4	3	3	2	2	2	NE
3 copies (N=13)	13	13	13	13	12	8	7	6	3	3	2	2	1	1
>=4 copies (N=5)	5	5	5	5	5	4	2	2	2	1	1	1	1	1
All Patients (N=26)	26	26	26	26	24	18	13	12	8	7	5	5	4	2
Patients censored														
2 copies (N=8)	0	0	0	0	1	2	4	4	5	5	6	6	6	NE
3 copies (N=13)	0	0	0	0	1	5	6	7	10	10	11	11	12	12
>=4 copies (N=5)	0	0	0	0	0	1	3	3	3	4	4	4	4	4
All Patients (N=26)	0	0	0	0	2	8	13	14	18	19	21	21	22	24

Clinical cut-off: 20FEB2023

Program: ..al_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/g_km.sas
Output: ..7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/g_km_DTH_IT_20FEB2023_40703.pdf
17AUG2023 12:09

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death or Permanent Ventilation (from enrollment)
MODEL: Unstratified Analysis
STUDY: BN40703
Time to Event Analysis (Efficacy)

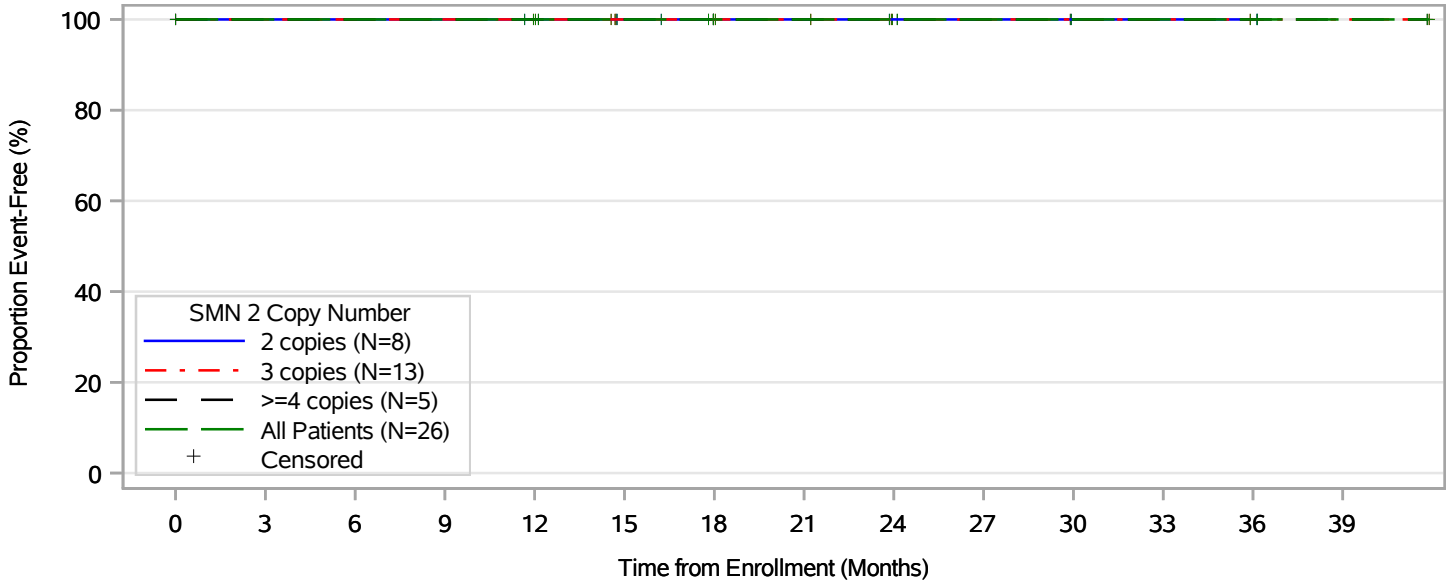
		Patients with 2 SMN2 copies (N=8)												Patients with 3 SMN2 copies (N=13)											
		Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
Name	Level	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
All	n/a	8	100.0	0	0	8	100.0	NE	NE	NE	NE	NE	NE	13	100.0	0	0	13	100.0	NE	NE	NE	NE	NE	NE

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_tte.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_tte_PVDTH_IT_20FEB2023_40703.xls
17AUG2023 12:07

Patients with >=4 SMN2 copies (N=5)												All Patients (N=26)											
Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
5	100.0	0	0	5	100.0	NE	NE	NE	NE	NE	NE	26	100.0	0	0	26	100.0	NE	NE	NE	NE	NE	NE

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death or Permanent Ventilation (from enrollment)
STUDY: BN40703



Patients at risk														
2 copies (N=8)	8	8	8	8	7	6	4	4	3	3	2	2	2	NE
3 copies (N=13)	13	13	13	13	12	8	7	6	3	3	2	2	1	1
>=4 copies (N=5)	5	5	5	5	5	4	2	2	2	1	1	1	1	1
All Patients (N=26)	26	26	26	26	24	18	13	12	8	7	5	5	4	2
Patients censored														
2 copies (N=8)	0	0	0	0	1	2	4	4	5	5	6	6	6	NE
3 copies (N=13)	0	0	0	0	1	5	6	7	10	10	11	11	12	12
>=4 copies (N=5)	0	0	0	0	0	1	3	3	3	4	4	4	4	4
All Patients (N=26)	0	0	0	0	2	8	13	14	18	19	21	21	22	24

Clinical cut-off: 20FEB2023

Program: ..al_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/g_km.sas
Output: ..16/BN40703/data_analysis/ACE_CSRFinal/prod/output/g_km_PVDTH_IT_20FEB2023_40703.pdf
17AUG2023 12:12

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Permanent Ventilation (from enrollment)
MODEL: Unstratified Analysis
STUDY: BN40703
Time to Event Analysis (Efficacy)

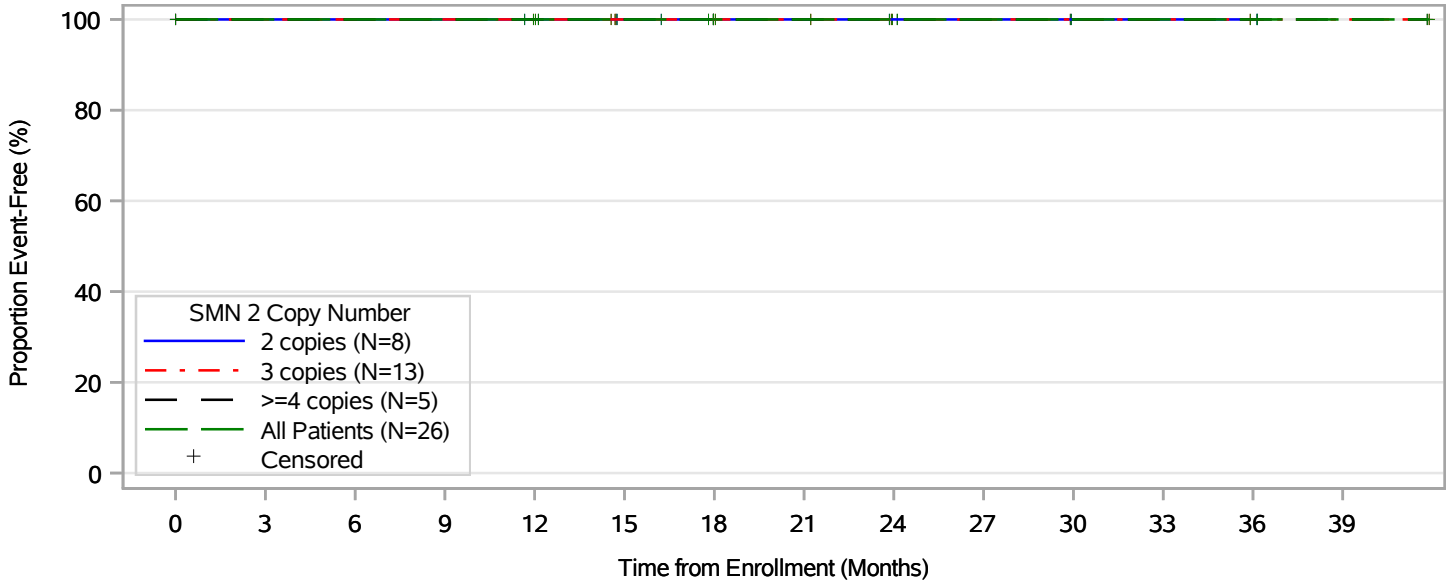
		Patients with 2 SMN2 copies (N=8)												Patients with 3 SMN2 copies (N=13)											
		Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
Name	Level	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
All	n/a	8	100.0	0	0	8	100.0	NE	NE	NE	NE	NE	NE	13	100.0	0	0	13	100.0	NE	NE	NE	NE	NE	NE

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_tte.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_tte_PV_IT_20FEB2023_40703.xls
17AUG2023 12:05

Patients with >=4 SMN2 copies (N=5)												All Patients (N=26)											
Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
5	100.0	0	0	5	100.0	NE	NE	NE	NE	NE	NE	26	100.0	0	0	26	100.0	NE	NE	NE	NE	NE	NE

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Permanent Ventilation (from enrollment)
STUDY: BN40703



Patients at risk														
2 copies (N=8)	8	8	8	8	7	6	4	4	3	3	2	2	2	NE
3 copies (N=13)	13	13	13	13	12	8	7	6	3	3	2	2	1	1
>=4 copies (N=5)	5	5	5	5	5	4	2	2	2	1	1	1	1	1
All Patients (N=26)	26	26	26	26	24	18	13	12	8	7	5	5	4	2
Patients censored														
2 copies (N=8)	0	0	0	0	1	2	4	4	5	5	6	6	6	NE
3 copies (N=13)	0	0	0	0	1	5	6	7	10	10	11	11	12	12
>=4 copies (N=5)	0	0	0	0	0	1	3	3	3	4	4	4	4	4
All Patients (N=26)	0	0	0	0	2	8	13	14	18	19	21	21	22	24

Clinical cut-off: 20FEB2023

Program: ..al_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/g_km.sas
Output: ..T7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/g_km_PV_IT_20FEB2023_40703.pdf
17AUG2023 12:10

POPULATION: Intent-to-Treat Population
ENDPOINT: Gross Motor Subtest: Total raw score
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	8	100.0	8	100.0	3.88	2.70	13	100.0	13	100.0	6.92	2.14	5	100.0	5	100.0	7.20	2.86	26	100.0	26	100.0	6.04	2.78
Week 4	n/a	8	100.0	8	100.0	6.38	4.63	13	100.0	13	100.0	10.62	2.93	5	100.0	5	100.0	10.00	2.92	26	100.0	26	100.0	9.19	3.90
Week 8	n/a	8	100.0	7	87.5	8.86	4.56	13	100.0	13	100.0	13.46	2.63	5	100.0	5	100.0	13.00	2.74	26	100.0	25	96.2	12.08	3.76
Week 16	n/a	8	100.0	8	100.0	15.13	1.81	13	100.0	13	100.0	18.54	2.37	5	100.0	4	80.0	17.50	1.00	26	100.0	25	96.2	17.28	2.51
Week 28	n/a	8	100.0	6	75.0	20.67	3.08	13	100.0	12	92.3	27.42	3.26	5	100.0	4	80.0	24.75	3.50	26	100.0	22	84.6	25.09	4.28
Week 40	n/a	8	100.0	7	87.5	23.29	10.78	13	100.0	13	100.0	35.38	4.05	5	100.0	3	60.0	30.67	2.31	26	100.0	23	88.5	31.09	8.45
Week 52	n/a	8	100.0	6	75.0	24.17	13.93	13	100.0	12	92.3	42.58	3.40	5	100.0	4	80.0	40.75	2.63	26	100.0	22	84.6	37.23	10.99
Week 64	n/a	7	87.5	7	100.0	30.43	10.21	12	92.3	10	83.3	47.50	1.78	4	80.0	4	100.0	43.50	4.04	23	88.5	21	91.3	41.05	9.83
Week 78	n/a	5	62.5	4	80.0	37.75	9.22	9	69.2	8	88.9	50.13	1.96	4	80.0	3	75.0	48.00	3.61	18	69.2	15	83.3	46.40	7.20
Week 92	n/a	4	50.0	4	100.0	40.00	11.28	7	53.8	5	71.4	52.80	2.77	4	80.0	2	50.0	51.00	0.00	15	57.7	11	73.3	47.82	8.95
Week 104	n/a	4	50.0	4	100.0	42.50	13.08	6	46.2	5	83.3	53.40	3.29	2	40.0	2	100.0	54.00	0.00	12	46.2	11	91.7	49.55	9.32
OLE Baseline	n/a	4	50.0	3	75.0	49.00	16.82	4	30.8	3	75.0	57.00	7.00	1	20.0	1	100.0	55.00	NE	9	34.6	7	77.8	53.29	11.28
OLE Week 26	n/a	2	25.0	2	100.0	59.50	6.36	2	15.4	2	100.0	63.50	3.54	1	20.0	1	100.0	61.00	NE	5	19.2	5	100.0	61.40	4.16
OLE Week 52	n/a	1	12.5	0	NE	NE	NE	2	15.4	1	50.0	67.00	NE	1	20.0	1	100.0	66.00	NE	4	15.4	2	50.0	66.50	0.71

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - absolute values
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_mean_GMSTRS_IT_20FEB2023_40703.xls
17AUG2023 12:04

POPULATION: Intent-to-Treat Population
ENDPOINT: Change from Baseline in Gross Motor Subtest: Total raw score up to Month 12
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	8	100.0	8	100.0	NE	NE	13	100.0	13	100.0	NE	NE	5	100.0	5	100.0	NE	NE	26	100.0	26	100.0	NE	NE
Week 4	n/a	8	100.0	8	100.0	2.50	3.46	13	100.0	13	100.0	3.69	3.20	5	100.0	5	100.0	2.80	2.17	26	100.0	26	100.0	3.15	3.06
Week 8	n/a	8	100.0	7	87.5	5.57	4.08	13	100.0	13	100.0	6.54	3.43	5	100.0	5	100.0	5.80	1.30	26	100.0	25	96.2	6.12	3.24
Week 16	n/a	8	100.0	8	100.0	11.25	1.91	13	100.0	13	100.0	11.62	3.43	5	100.0	4	80.0	11.25	2.22	26	100.0	25	96.2	11.44	2.75
Week 28	n/a	8	100.0	6	75.0	17.17	4.45	13	100.0	12	92.3	20.50	2.75	5	100.0	4	80.0	17.50	4.93	26	100.0	22	84.6	19.05	3.85
Week 40	n/a	8	100.0	7	87.5	19.14	9.81	13	100.0	13	100.0	28.46	4.45	5	100.0	3	60.0	24.67	2.08	26	100.0	23	88.5	25.13	7.44
Week 52	n/a	8	100.0	6	75.0	19.50	12.53	13	100.0	12	92.3	35.25	3.52	5	100.0	4	80.0	32.75	4.43	26	100.0	22	84.6	30.50	9.75

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - change from baseline
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_mean_chg_GMSTRS_IT_20FEB2023_40703.xls
17AUG2023 12:07

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants sitting without support for 5 seconds (confirmed) at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	13	100.0	77.2	100.0	5	100.0	5	100.0	56.6	100.0	26	100.0	25	96.2	81.1	99.3

95% CI based on Wilson Scores.
Patients with missing Week 52 response are considered as non-responders.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_SIT05_IT_20FEB2023_40703.xls
18AUG2023 14:18

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants sitting without support for 30 seconds (confirmed) at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	9	69.2	42.4	87.3	5	100.0	5	100.0	56.6	100.0	26	100.0	21	80.8	62.1	91.5

95% CI based on Wilson Scores.
Patients with missing Week 52 response are considered as non-responders.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_SIT30_IT_20FEB2023_40703.xls
18AUG2023 14:20

POPULATION: Intent-to-Treat Population
ENDPOINT: CHOP-INTEND total score
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	8	100.0	8	100.0	44.88	6.56	13	100.0	13	100.0	53.77	5.76	5	100.0	5	100.0	48.60	3.44	26	100.0	26	100.0	50.04	6.79
Week 4	n/a	8	100.0	8	100.0	43.00	9.75	13	100.0	13	100.0	57.08	4.77	5	100.0	5	100.0	54.20	1.64	26	100.0	26	100.0	52.19	8.85
Week 8	n/a	8	100.0	7	87.5	50.14	6.89	13	100.0	13	100.0	58.15	4.08	5	100.0	5	100.0	55.80	3.35	26	100.0	25	96.2	55.44	5.85
Week 16	n/a	8	100.0	8	100.0	55.63	7.19	13	100.0	13	100.0	61.54	1.71	5	100.0	5	100.0	59.20	4.97	26	100.0	26	100.0	59.27	5.17
Week 28	n/a	8	100.0	8	100.0	60.63	2.77	13	100.0	12	92.3	63.75	0.62	5	100.0	4	80.0	61.50	2.52	26	100.0	24	92.3	62.33	2.35
Week 40	n/a	8	100.0	8	100.0	57.38	8.30	13	100.0	13	100.0	63.62	0.96	5	100.0	5	100.0	61.40	5.81	26	100.0	26	100.0	61.27	5.73
Week 52	n/a	8	100.0	8	100.0	53.38	11.48	13	100.0	13	100.0	63.69	0.75	5	100.0	4	80.0	62.75	1.89	26	100.0	25	96.2	60.24	7.90
Week 64	n/a	7	87.5	5	71.4	57.00	8.69	12	92.3	3	25.0	64.00	0.00	4	80.0	2	50.0	64.00	0.00	23	88.5	10	43.5	60.50	6.87
Week 78	n/a	5	62.5	0	NE	NE	NE	9	69.2	0	NE	NE	NE	4	80.0	0	NE	NE	NE	18	69.2	0	NE	NE	NE
Week 92	n/a	4	50.0	0	NE	NE	NE	7	53.8	0	NE	NE	NE	4	80.0	0	NE	NE	NE	15	57.7	0	NE	NE	NE
Week 104	n/a	4	50.0	0	NE	NE	NE	6	46.2	0	NE	NE	NE	2	40.0	0	NE	NE	NE	12	46.2	0	NE	NE	NE
OLE Baseline	n/a	4	50.0	0	NE	NE	NE	4	30.8	0	NE	NE	NE	1	20.0	0	NE	NE	NE	9	34.6	0	NE	NE	NE
OLE Week 26	n/a	2	25.0	0	NE	NE	NE	2	15.4	0	NE	NE	NE	1	20.0	0	NE	NE	NE	5	19.2	0	NE	NE	NE
OLE Week 52	n/a	1	12.5	0	NE	NE	NE	2	15.4	0	NE	NE	NE	1	20.0	0	NE	NE	NE	4	15.4	0	NE	NE	NE

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - absolute values
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_mean_CHOP_IT_20FEB2023_40703.xls
30AUG2023 10:16

POPULATION: Intent-to-Treat Population
ENDPOINT: Change from Baseline in CHOP-INTEND total score up to Month 12
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	8	100.0	8	100.0	NE	NE	13	100.0	13	100.0	NE	NE	5	100.0	5	100.0	NE	NE	26	100.0	26	100.0	NE	NE
Week 4	n/a	8	100.0	8	100.0	-1.88	6.69	13	100.0	13	100.0	3.31	4.94	5	100.0	5	100.0	5.60	4.16	26	100.0	26	100.0	2.15	5.94
Week 8	n/a	8	100.0	7	87.5	5.29	6.63	13	100.0	13	100.0	4.38	6.45	5	100.0	5	100.0	7.20	4.92	26	100.0	25	96.2	5.20	6.08
Week 16	n/a	8	100.0	8	100.0	10.75	5.52	13	100.0	13	100.0	7.77	6.52	5	100.0	5	100.0	10.60	7.92	26	100.0	26	100.0	9.23	6.42
Week 28	n/a	8	100.0	8	100.0	15.75	6.07	13	100.0	12	92.3	9.33	5.50	5	100.0	4	80.0	13.25	5.85	26	100.0	24	92.3	12.13	6.24
Week 40	n/a	8	100.0	8	100.0	12.50	5.68	13	100.0	13	100.0	9.85	5.98	5	100.0	5	100.0	12.80	8.23	26	100.0	26	100.0	11.23	6.25
Week 52	n/a	8	100.0	8	100.0	8.50	9.70	13	100.0	13	100.0	9.92	5.62	5	100.0	4	80.0	14.75	4.99	26	100.0	25	96.2	10.24	7.13

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - change from baseline
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_mean_chg_CHOP_IT_20FEB2023_40703.xls
17AUG2023 12:08

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of patients who achieve a score of 40 or higher in the CHOP-INTEND at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	6	75.0	40.9	92.9	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	23	92.0	75.0	97.8

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_CHOP40_IT_20FEB2023_40703.xls
17AUG2023 15:45

POPULATION: Intent-to-Treat Population
ENDPOINT: HINE-2 total score
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	8	100.0	8	100.0	2.13	1.46	13	100.0	13	100.0	3.00	1.41	5	100.0	5	100.0	2.00	1.41	26	100.0	26	100.0	2.54	1.45
Week 4	n/a	8	100.0	8	100.0	2.13	1.46	13	100.0	13	100.0	4.54	1.76	5	100.0	5	100.0	4.00	0.71	26	100.0	26	100.0	3.69	1.83
Week 8	n/a	8	100.0	7	87.5	3.29	1.25	13	100.0	13	100.0	5.46	2.15	5	100.0	5	100.0	4.80	1.48	26	100.0	25	96.2	4.72	1.99
Week 16	n/a	8	100.0	8	100.0	5.50	1.85	13	100.0	13	100.0	9.08	3.12	5	100.0	5	100.0	8.00	2.83	26	100.0	26	100.0	7.77	3.08
Week 28	n/a	8	100.0	8	100.0	11.13	3.64	13	100.0	12	92.3	16.75	3.74	5	100.0	5	100.0	15.80	3.83	26	100.0	25	96.2	14.76	4.40
Week 40	n/a	8	100.0	8	100.0	14.50	6.02	13	100.0	12	92.3	21.92	1.78	5	100.0	5	100.0	20.00	3.08	26	100.0	25	96.2	19.16	4.98
Week 52	n/a	8	100.0	8	100.0	17.13	7.28	13	100.0	13	100.0	24.92	1.44	5	100.0	4	80.0	24.25	2.06	26	100.0	25	96.2	22.32	5.51
Week 64	n/a	7	87.5	7	100.0	19.50	4.72	12	92.3	11	91.7	25.91	0.30	4	80.0	4	100.0	25.25	1.50	23	88.5	22	95.7	23.57	4.09
Week 78	n/a	5	62.5	4	80.0	23.75	2.63	9	69.2	8	88.9	25.88	0.35	4	80.0	3	75.0	26.00	0.00	18	69.2	15	83.3	25.33	1.59
Week 92	n/a	4	50.0	4	100.0	23.00	3.83	7	53.8	5	71.4	26.00	0.00	4	80.0	2	50.0	26.00	0.00	15	57.7	11	73.3	24.91	2.59
Week 104	n/a	4	50.0	4	100.0	23.75	3.30	6	46.2	4	66.7	26.00	0.00	2	40.0	2	100.0	26.00	0.00	12	46.2	10	83.3	25.10	2.23
OLE Baseline	n/a	4	50.0	2	50.0	26.00	0.00	4	30.8	0	NE	NE	NE	1	20.0	1	100.0	26.00	NE	9	34.6	3	33.3	26.00	0.00
OLE Week 26	n/a	2	25.0	2	100.0	26.00	0.00	2	15.4	0	NE	NE	NE	1	20.0	0	NE	NE	NE	5	19.2	2	40.0	26.00	0.00
OLE Week 52	n/a	1	12.5	0	NE	NE	NE	2	15.4	0	NE	NE	NE	1	20.0	1	100.0	26.00	NE	4	15.4	1	25.0	26.00	NE

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - absolute values
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_mean_HINE_IT_20FEB2023_40703.xls
30AUG2023 10:17

POPULATION: Intent-to-Treat Population
ENDPOINT: Change from Baseline in HINE-2 total score up to Month 12
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	8	100.0	8	100.0	NE	NE	13	100.0	13	100.0	NE	NE	5	100.0	5	100.0	NE	NE	26	100.0	26	100.0	NE	NE
Week 4	n/a	8	100.0	8	100.0	0.00	1.77	13	100.0	13	100.0	1.54	1.33	5	100.0	5	100.0	2.00	1.58	26	100.0	26	100.0	1.15	1.67
Week 8	n/a	8	100.0	7	87.5	1.00	0.82	13	100.0	13	100.0	2.46	1.90	5	100.0	5	100.0	2.80	2.17	26	100.0	25	96.2	2.12	1.81
Week 16	n/a	8	100.0	8	100.0	3.38	2.20	13	100.0	13	100.0	6.08	3.04	5	100.0	5	100.0	6.00	3.87	26	100.0	26	100.0	5.23	3.13
Week 28	n/a	8	100.0	8	100.0	9.00	4.00	13	100.0	12	92.3	13.83	3.56	5	100.0	5	100.0	13.80	5.17	26	100.0	25	96.2	12.28	4.50
Week 40	n/a	8	100.0	8	100.0	12.38	6.28	13	100.0	12	92.3	19.17	2.08	5	100.0	5	100.0	18.00	4.30	26	100.0	25	96.2	16.76	5.12
Week 52	n/a	8	100.0	8	100.0	15.00	7.67	13	100.0	13	100.0	21.92	2.10	5	100.0	4	80.0	22.75	2.63	26	100.0	25	96.2	19.84	5.64

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - change from baseline
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_mean_chg_HINE_IT_20FEB2023_40703.xls
17AUG2023 12:09

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Head Control: Wobbles

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	2	8.0	2.2	25.0

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Head Control: All the time maintained upright

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	6	75.0	40.9	92.9	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	23	92.0	75.0	97.8

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Sitting: Cannot sit

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Sitting: Stable sit

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Sitting: Pivots (rotates)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	6	75.0	40.9	92.9	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	23	92.0	75.0	97.8

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Sitting Grouped

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	24	96.0	80.5	99.3

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Voluntary grasp: Index finger and thumb but immature grasp

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	3	23.1	8.2	50.3	4	80.0	1	25.0	4.6	69.9	25	96.2	6	24.0	11.5	43.4

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Voluntary grasp: Pincer grasp

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	6	75.0	40.9	92.9	13	100.0	10	76.9	49.7	91.8	4	80.0	3	75.0	30.1	95.4	25	96.2	19	76.0	56.6	88.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Ability to Kick (in Supine): Kicks horizontally; legs do not lift

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	2	8.0	2.2	25.0

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Ability to Kick (in Supine): Touches leg

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Ability to Kick (in Supine): Touches toes

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Ability to Kick (in Supine): Upwards (vertically)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	5	62.5	30.6	86.3	13	100.0	12	92.3	66.7	98.6	4	80.0	4	100.0	51.0	100.0	25	96.2	21	84.0	65.3	93.6

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Rolling: No rolling

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Rolling: Rolling to side

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Rolling: Prone to supine

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	2	8.0	2.2	25.0

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Rolling: Supine to prone

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	4	50.0	21.5	78.5	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	21	84.0	65.3	93.6

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Rolling Grouped

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	24	96.0	80.5	99.3

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Crawling: Does not lift head

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	2	8.0	2.2	25.0

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Crawling: On elbow

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Crawling: On outstretched hand

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Crawling: Crawling flat on abdomen

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	1	4.0	0.7	19.5

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Crawling: Crawling on hands and knees

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	20	80.0	60.9	91.1

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Standing: Does not support weight

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	4	50.0	21.5	78.5	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	25	96.2	4	16.0	6.4	34.7

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Standing: Stands with support

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	3	23.1	8.2	50.3	4	80.0	2	50.0	15.0	85.0	25	96.2	8	32.0	17.2	51.6

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Standing: Stands unaided

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	10	76.9	49.7	91.8	4	80.0	2	50.0	15.0	85.0	25	96.2	13	52.0	33.5	70.0

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Standing Grouped

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	4	50.0	21.5	78.5	13	100.0	13	100.0	77.2	100.0	4	80.0	4	100.0	51.0	100.0	25	96.2	21	84.0	65.3	93.6

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Walking: Bouncing

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	87.5	4	57.1	25.0	84.2	13	100.0	1	7.7	1.4	33.3	4	80.0	1	25.0	4.6	69.9	24	92.3	6	25.0	12.0	44.9

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Walking: Cannot test

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	87.5	2	28.6	8.2	64.1	13	100.0	0	0.0	0.0	22.8	4	80.0	0	0.0	0.0	49.0	24	92.3	2	8.3	2.3	25.8

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Walking: Cruising (walks holding on)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	87.5	0	0.0	0.0	35.4	13	100.0	3	23.1	8.2	50.3	4	80.0	1	25.0	4.6	69.9	24	92.3	4	16.7	6.7	35.9

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703

Walking: Walking Independently

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	87.5	1	14.3	2.6	51.3	13	100.0	9	69.2	42.4	87.3	4	80.0	2	50.0	15.0	85.0	24	92.3	12	50.0	31.4	68.6

95% CI based on Wilson Scores.
Only patients with assessment performed at Week 52 are used for analysis.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_hine_grp_IT_20FEB2023_40703.xls
18AUG2023 14:45

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of motor milestone responders (HINE-2) at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	13	100.0	77.2	100.0	5	100.0	4	80.0	37.6	96.4	26	100.0	24	92.3	75.9	97.9

95% CI based on Wilson Scores.
Patients with missing Week 52 response are considered as non-responders.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_MMR_IT_20FEB2023_40703.xls
18AUG2023 14:11

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to swallow at Baseline
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	8	100.0	67.6	100.0	13	100.0	13	100.0	77.2	100.0	5	100.0	5	100.0	56.6	100.0	26	100.0	26	100.0	87.1	100.0

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_bl_ASLW_IT_20FEB2023_40703.xls
07SEP2023 11:24

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to swallow at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	8	100.0	67.6	100.0	13	100.0	13	100.0	77.2	100.0	5	100.0	5	100.0	56.6	100.0	26	100.0	26	100.0	87.1	100.0

95% CI based on Wilson Scores.
Patients with missing Week 52 response are considered as non-responders.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_ASLW_IT_20FEB2023_40703.xls
18AUG2023 14:09

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to feed orally at Baseline
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	8	100.0	67.6	100.0	13	100.0	13	100.0	77.2	100.0	5	100.0	5	100.0	56.6	100.0	26	100.0	26	100.0	87.1	100.0

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_bl_FEED_IT_20FEB2023_40703.xls
07SEP2023 11:25

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to feed orally at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	13	100.0	77.2	100.0	5	100.0	5	100.0	56.6	100.0	26	100.0	25	96.2	81.1	99.3

95% CI based on Wilson Scores.
Patients with missing Week 52 response are considered as non-responders.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_resp_FEED_IT_20FEB2023_40703.xls
18AUG2023 14:08

POPULATION: Intent-to-Treat Population
ENDPOINT: Number of Hospitalizations (for any reason) per Patient-Year at Month 12.
MODEL: --
STUDY: BN40703

			Patients with 2 SMN2 copies (N=8)	Patients with 3 SMN2 copies (N=13)	Patients with >=4 SMN2 copies (N=5)	All Patients (N=26)
Name	Level		Statistics	Statistics	Statistics	Statistics
All	n/a	Total patient-years at risk	7.9	12.9	5	25.9
		Number of hospitalizations	1	0	1	2
		Number of hospitalizations per patient-year	0.13	NE	0.2	0.08
		95% CI	(0.0, 0.7)	NE	(0.0, 1.1)	(0.0, 0.3)

Total patient-years at risk is the sum over all patients of the time intervals (in years) from the start of study treatment to the earliest date of study withdrawal or completion of 12 months of treatment (Month 12 visit).
Includes all hospital admissions observed by Month 12 which span at least two days.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/program/t_eff_hosp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_hosp_IT_20FEB2023_40703.xls
17AUG2023 12:26

POPULATION: Intent-to-Treat Population
ENDPOINT: Number of Nights Admitted to Hospital per Infant at Month 12.
MODEL: --
STUDY: BN40703

	Name	Level		Patients with 2 SMN2 copies (N=8)	Patients with >=4 SMN2 copies (N=5)	All Patients (N=26)
Number of nights per infant	All	n/a	n	1	1	2
			Mean (SD)	4.00 (NE)	10.00 (NE)	7.00 (4.24)
			Median	4	10	7
			IQR	4.0 - 4.0	10.0 - 10.0	4.0 - 10.0
			Min - Max	4.0 - 4.0	10.0 - 10.0	4.0 - 10.0
Number of nights per infant (categorical)	All	n/a	n	1	1	2
			4-7 Nights	1 (100%)	0	1 (50.0%)
			8-14 Nights	0	1 (100%)	1 (50.0%)

Includes all hospital admissions observed by Month 12 which span at least two days.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_hosp_nights.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_eff_hosp_nights_IT_20FEB2023_40703.xls
17AUG2023 12:27

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	8	100.0	67.6	100.0	13	100.0	12	92.3	66.7	98.6	5	100.0	4	80.0	37.6	96.4	26	100.0	24	92.3	75.9	97.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_AE_SE_20FEB2023_40703.xls
17AUG2023 12:35

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	12	92.3	66.7	98.6	5	100.0	4	80.0	37.6	96.4	26	100.0	23	88.5	71.0	96.0

95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_AE_EXCL_SE_20FEB2023_40703.xls
17AUG2023 13:01

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	8	100.0	67.6	100.0	13	100.0	12	92.3	66.7	98.6	5	100.0	4	80.0	37.6	96.4	26	100.0	24	92.3	75.9	97.9

95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_AE_EXCLN_SE_20FEB2023_40703.xls
17AUG2023 13:27

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_SAE_SE_20FEB2023_40703.xls
17AUG2023 12:45

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_SAE_EXCL_SE_20FEB2023_40703.xls
17AUG2023 13:14

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_SAE_EXCLN_SE_20FEB2023_40703.xls
17AUG2023 13:40

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs leading to any Study Treatment Discontinuation
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WDSTAE_SE_20FEB2023_40703.xls
17AUG2023 12:47

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1/2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	5	62.5	30.6	86.3	13	100.0	10	76.9	49.7	91.8	5	100.0	3	60.0	23.1	88.2	26	100.0	18	69.2	50.0	83.5

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR12AE_SE_20FEB2023_40703.xls
17AUG2023 12:39

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR1AE_SE_20FEB2023_40703.xls
17AUG2023 12:36

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	8	61.5	35.5	82.3	5	100.0	2	40.0	11.8	76.9	26	100.0	13	50.0	32.1	67.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR2AE_SE_20FEB2023_40703.xls
17AUG2023 12:37

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR35AE_SE_20FEB2023_40703.xls
17AUG2023 12:40

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5 Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR35AE_EXCL_SE_20FEB2023_40703.xls
17AUG2023 13:08

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5 Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR35AE_EXCLN_SE_20FEB2023_40703.xls
17AUG2023 13:34

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR3AE_SE_20FEB2023_40703.xls
17AUG2023 12:41

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3 Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR3AE_EXCL_SE_20FEB2023_40703.xls
17AUG2023 13:09

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3 Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR3AE_EXCLN_SE_20FEB2023_40703.xls
17AUG2023 13:35

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR4AE_SE_20FEB2023_40703.xls
17AUG2023 12:42

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4 Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR4AE_EXCL_SE_20FEB2023_40703.xls
17AUG2023 13:11

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4 Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR4AE_EXCLN_SE_20FEB2023_40703.xls
17AUG2023 13:37

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death)
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR5AE_SE_20FEB2023_40703.xls
17AUG2023 12:44

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death) Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR5AE_EXCL_SE_20FEB2023_40703.xls
17AUG2023 13:12

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death) Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WGR5AE_EXCLN_SE_20FEB2023_40703.xls
17AUG2023 13:38

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Blood and lymphatic system disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Blood and lymphatic system disorders	Anaemia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Congenital, familial and genetic disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Congenital, familial and genetic disorders	Cryptorchism	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	3	11.5	4.0	29.0
Eye disorders	Cystoid macular oedema	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Retinal pigmentation	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Retinal vascular disorder	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders		n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	9	69.2	42.4	87.3	5	100.0	3	60.0	23.1	88.2	26	100.0	14	53.8	35.5	71.2
Gastrointestinal disorders	Abdominal pain	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Gastrointestinal disorders	Constipation	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	6	23.1	11.0	42.1
Gastrointestinal disorders	Diarrhoea	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	6	23.1	11.0	42.1
Gastrointestinal disorders	Dyspepsia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Gastrointestinal pain	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Gastrooesophageal reflux disease	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Regurgitation	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Teething	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	6	46.2	23.2	70.9	5	100.0	2	40.0	11.8	76.9	26	100.0	10	38.5	22.4	57.5
Gastrointestinal disorders	Vomiting	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	5	19.2	8.5	37.9
General disorders and administration site conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	7	26.9	13.7	46.1
General disorders and administration site conditions	Malaise	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
General disorders and administration site conditions	Pyrexia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	7	26.9	13.7	46.1
Infections and infestations		n/a	8	100.0	8	100.0	67.6	100.0	13	100.0	11	84.6	57.8	95.7	5	100.0	4	80.0	37.6	96.4	26	100.0	23	88.5	71.0	96.0
Infections and infestations	Bronchiolitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Bronchitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	COVID-19	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	7	53.8	29.1	76.8	5	100.0	0	0.0	0.0	43.4	26	100.0	9	34.6	19.4	53.8
Infections and infestations	Conjunctivitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Croup infectious	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Cytomegalovirus infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Ear infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Exanthema subitum	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastroenteritis	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	2	15.4	4.3	42.2	5	100.0	3	60.0	23.1	88.2	26	100.0	7	26.9	13.7	46.1
Infections and infestations	Gastroenteritis norovirus	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastrointestinal viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Hand-foot-and-mouth disease	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Impetigo	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Influenza	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Nasopharyngitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Infections and infestations	Oral candidiasis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Otitis media acute	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory syncytial virus bronchitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory syncytial virus infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection bacterial	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection viral	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	4	15.4	6.2	33.5
Infections and infestations	Rhinitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	5	19.2	8.5	37.9
Infections and infestations	Skin infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Suspected COVID-19	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Tonsillitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Upper respiratory tract infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Urinary tract infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Varicella	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Viral rash	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Vulvovaginitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	6	46.2	23.2	70.9	5	100.0	1	20.0	3.6	62.4	26	100.0	10	38.5	22.4	57.5

Injury, poisoning and procedural complications	Accidental overdose	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	3	23.1	8.2	50.3	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Injury, poisoning and procedural complications	Arthropod bite	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Contusion	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Expired product administered	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Injury, poisoning and procedural complications	Fall	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Femur fracture	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Incorrect dose administered	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Injury, poisoning and procedural complications	Intercepted medication error	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Limb injury	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Overdose	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Soft tissue injury	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Underdose	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Investigations		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Alanine aminotransferase increased	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Aspartate aminotransferase increased	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Cardiac murmur	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	0	0.0	0.0	43.4	26	100.0	4	15.4	6.2	33.5
Metabolism and nutrition disorders	Decreased appetite	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Hyperphosphatasaemia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Hypoglycaemia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Iron deficiency	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Vitamin D deficiency	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Musculoskeletal and connective tissue disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Musculoskeletal and connective tissue disorders	Pain in extremity	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Pregnancy, puerperium and perinatal conditions	Jaundice neonatal	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions	Umbilical granuloma	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders		n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	7	26.9	13.7	46.1
Respiratory, thoracic and mediastinal disorders	Cough	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
Respiratory, thoracic and mediastinal disorders	Epistaxis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Respiratory, thoracic and mediastinal disorders	Nasal congestion	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Respiratory, thoracic and mediastinal disorders	Rhinitis allergic	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders	Rhinorrhoea	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders		n/a	8	100.0	4	50.0	21.5	78.5	13	100.0	6	46.2	23.2	70.9	5	100.0	4	80.0	37.6	96.4	26	100.0	14	53.8	35.5	71.2
Skin and subcutaneous tissue disorders	Dermatitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis atopic	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders	Dermatitis contact	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis diaper	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Eczema	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	1	20.0	3.6	62.4	26	100.0	6	23.1	11.0	42.1
Skin and subcutaneous tissue disorders	Erythema	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Papule	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders	Rash	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Skin and subcutaneous tissue disorders	Rash maculo-papular	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Skin discolouration	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9

95% CI based on Wilson Scores.

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_AE_SE_20FEB2023_40703.xls
17AUG2023 13:57

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Gastrointestinal disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Constipation	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Gastroenteritis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Urinary tract infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Femur fracture	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Soft tissue injury	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions	Jaundice neonatal	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_SAE_SE_20FEB2023_40703.xls
17AUG2023 14:07

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs leading to any Study Treatment Discontinuation
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WDSTAE_SE_20FEB2023_40703.xls
17AUG2023 14:08

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1/2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Blood and lymphatic system disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Blood and lymphatic system disorders	Anaemia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Congenital, familial and genetic disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Congenital, familial and genetic disorders	Cryptorchism	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Eye disorders	Retinal pigmentation	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Retinal vascular disorder	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	9	69.2	42.4	87.3	5	100.0	3	60.0	23.1	88.2	26	100.0	13	50.0	32.1	67.9
Gastrointestinal disorders	Abdominal pain	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Gastrointestinal disorders	Constipation	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Gastrointestinal disorders	Diarrhoea	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	6	23.1	11.0	42.1
Gastrointestinal disorders	Dyspepsia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Gastrointestinal pain	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Gastrooesophageal reflux disease	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Regurgitation	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Teething	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	6	46.2	23.2	70.9	5	100.0	2	40.0	11.8	76.9	26	100.0	10	38.5	22.4	57.5
Gastrointestinal disorders	Vomiting	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	5	19.2	8.5	37.9
General disorders and administration site conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	7	26.9	13.7	46.1
General disorders and administration site conditions	Malaise	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
General disorders and administration site conditions	Pyrexia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	7	26.9	13.7	46.1
Infections and infestations		n/a	8	100.0	7	87.5	52.9	97.8	13	100.0	10	76.9	49.7	91.8	5	100.0	3	60.0	23.1	88.2	26	100.0	20	76.9	57.9	89.0
Infections and infestations	Bronchiolitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Bronchitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	COVID-19	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	7	53.8	29.1	76.8	5	100.0	0	0.0	0.0	43.4	26	100.0	9	34.6	19.4	53.8
Infections and infestations	Conjunctivitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Croup infectious	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Cytomegalovirus infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Ear infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Exanthema subitum	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastroenteritis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	3	60.0	23.1	88.2	26	100.0	6	23.1	11.0	42.1
Infections and infestations	Gastrointestinal viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Hand-foot-and-mouth disease	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Impetigo	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Influenza	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Nasopharyngitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Infections and infestations	Oral candidiasis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Otitis media acute	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory syncytial virus bronchitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory syncytial virus infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection bacterial	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection viral	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	4	15.4	6.2	33.5
Infections and infestations	Rhinitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	5	19.2	8.5	37.9
Infections and infestations	Skin infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Suspected COVID-19	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Tonsillitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Upper respiratory tract infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Varicella	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Viral rash	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Vulvovaginitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	0	0.0	0.0	43.4	26	100.0	5	19.2	8.5	37.9
Injury, poisoning and procedural complications	Accidental overdose	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Injury, poisoning and procedural complications	Arthropod bite	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9

Injury, poisoning and procedural complications	Contusion	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Expired product administered	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Injury, poisoning and procedural complications	Fall	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Limb injury	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Soft tissue injury	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Alanine aminotransferase increased	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Aspartate aminotransferase increased	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Cardiac murmur	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	0	0.0	0.0	43.4	26	100.0	4	15.4	6.2	33.5
Metabolism and nutrition disorders	Decreased appetite	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Hyperphosphatasaemia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Hypoglycaemia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Iron deficiency	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Vitamin D deficiency	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Musculoskeletal and connective tissue disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Musculoskeletal and connective tissue disorders	Pain in extremity	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Pregnancy, puerperium and perinatal conditions	Jaundice neonatal	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions	Umbilical granuloma	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders		n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	7	26.9	13.7	46.1
Respiratory, thoracic and mediastinal disorders	Cough	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
Respiratory, thoracic and mediastinal disorders	Epistaxis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Respiratory, thoracic and mediastinal disorders	Nasal congestion	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Respiratory, thoracic and mediastinal disorders	Rhinitis allergic	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders	Rhinorrhoea	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders		n/a	8	100.0	4	50.0	21.5	78.5	13	100.0	6	46.2	23.2	70.9	5	100.0	4	80.0	37.6	96.4	26	100.0	14	53.8	35.5	71.2
Skin and subcutaneous tissue disorders	Dermatitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis atopic	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders	Dermatitis contact	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis diaper	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Eczema	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	4	30.8	12.7	57.6	5	100.0	1	20.0	3.6	62.4	26	100.0	6	23.1	11.0	42.1
Skin and subcutaneous tissue disorders	Erythema	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Papule	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders	Rash	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Skin and subcutaneous tissue disorders	Rash maculo-papular	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Skin discolouration	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR12AE_SE_20FEB2023_40703.xls
17AUG2023 14:01

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Blood and lymphatic system disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Blood and lymphatic system disorders	Anaemia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Congenital, familial and genetic disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Congenital, familial and genetic disorders	Cryptorchism	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Retinal vascular disorder	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	8	61.5	35.5	82.3	5	100.0	1	20.0	3.6	62.4	26	100.0	10	38.5	22.4	57.5
Gastrointestinal disorders	Abdominal pain	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Constipation	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Gastrointestinal disorders	Diarrhoea	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	4	30.8	12.7	57.6	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Gastrointestinal disorders	Dyspepsia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Gastrointestinal pain	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Gastrooesophageal reflux disease	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Regurgitation	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Teething	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	6	46.2	23.2	70.9	5	100.0	0	0.0	0.0	43.4	26	100.0	8	30.8	16.5	50.0
Gastrointestinal disorders	Vomiting	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
General disorders and administration site conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
General disorders and administration site conditions	Malaise	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
General disorders and administration site conditions	Pyrexia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations		n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	4	30.8	12.7	57.6	5	100.0	1	20.0	3.6	62.4	26	100.0	7	26.9	13.7	46.1
Infections and infestations	Bronchitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	COVID-19	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	3	23.1	8.2	50.3	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Conjunctivitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Cytomegalovirus infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastroenteritis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	4	15.4	6.2	33.5
Infections and infestations	Gastrointestinal viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Nasopharyngitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Oral candidiasis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Otitis media acute	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection viral	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	4	15.4	6.2	33.5
Infections and infestations	Rhinitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Skin infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Tonsillitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Varicella	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Viral rash	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	0	0.0	0.0	43.4	26	100.0	4	15.4	6.2	33.5
Injury, poisoning and procedural complications	Accidental overdose	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Arthropod bite	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Contusion	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Expired product administered	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Injury, poisoning and procedural complications	Fall	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Limb injury	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Soft tissue injury	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9
Investigations	Cardiac murmur	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	3	23.1	8.2	50.3	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Metabolism and nutrition disorders	Hyperphosphatasaemia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Hypoglycaemia	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9

Metabolism and nutrition disorders	Iron deficiency	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Vitamin D deficiency	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Musculoskeletal and connective tissue disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Musculoskeletal and connective tissue disorders	Pain in extremity	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Pregnancy, puerperium and perinatal conditions	Jaundice neonatal	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Pregnancy, puerperium and perinatal conditions	Umbilical granuloma	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders		n/a	8	100.0	3	37.5	13.7	69.4	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	6	23.1	11.0	42.1
Respiratory, thoracic and mediastinal disorders	Cough	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Respiratory, thoracic and mediastinal disorders	Epistaxis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Respiratory, thoracic and mediastinal disorders	Nasal congestion	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	3	23.1	8.2	50.3	5	100.0	1	20.0	3.6	62.4	26	100.0	5	19.2	8.5	37.9
Respiratory, thoracic and mediastinal disorders	Rhinitis allergic	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders	Rhinorrhoea	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders		n/a	8	100.0	4	50.0	21.5	78.5	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	8	30.8	16.5	50.0
Skin and subcutaneous tissue disorders	Dermatitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis atopic	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis contact	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Eczema	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	2	15.4	4.3	42.2	5	100.0	0	0.0	0.0	43.4	26	100.0	3	11.5	4.0	29.0
Skin and subcutaneous tissue disorders	Erythema	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Papule	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders	Rash	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Skin and subcutaneous tissue disorders	Rash maculo-papular	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Skin discolouration	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR1AE_SE_20FEB2023_40703.xls
17AUG2023 13:58

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Blood and lymphatic system disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Blood and lymphatic system disorders	Anaemia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Retinal pigmentation	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	2	40.0	11.8	76.9	26	100.0	3	11.5	4.0	29.0
Gastrointestinal disorders	Abdominal pain	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Diarrhoea	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Teething	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Gastrointestinal disorders	Vomiting	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
General disorders and administration site conditions		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	4	15.4	6.2	33.5
General disorders and administration site conditions	Pyrexia	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	2	40.0	11.8	76.9	26	100.0	4	15.4	6.2	33.5
Infections and infestations		n/a	8	100.0	5	62.5	30.6	86.3	13	100.0	6	46.2	23.2	70.9	5	100.0	2	40.0	11.8	76.9	26	100.0	13	50.0	32.1	67.9
Infections and infestations	Bronchiolitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	COVID-19	n/a	8	100.0	2	25.0	7.1	59.1	13	100.0	4	30.8	12.7	57.6	5	100.0	0	0.0	0.0	43.4	26	100.0	6	23.1	11.0	42.1
Infections and infestations	Conjunctivitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Croup infectious	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Ear infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Exanthema subitum	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastroenteritis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Gastrointestinal viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Hand-foot-and-mouth disease	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Impetigo	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Influenza	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Nasopharyngitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Oral candidiasis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory syncytial virus bronchitis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory syncytial virus infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Respiratory tract infection bacterial	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Rhinitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	2	40.0	11.8	76.9	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Suspected COVID-19	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Upper respiratory tract infection	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	2	7.7	2.1	24.1
Infections and infestations	Varicella	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Viral infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Vulvovaginitis	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Accidental overdose	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Alanine aminotransferase increased	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Investigations	Aspartate aminotransferase increased	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Metabolism and nutrition disorders	Decreased appetite	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Respiratory, thoracic and mediastinal disorders	Cough	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders		n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	4	30.8	12.7	57.6	5	100.0	2	40.0	11.8	76.9	26	100.0	6	23.1	11.0	42.1
Skin and subcutaneous tissue disorders	Dermatitis atopic	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Dermatitis diaper	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Skin and subcutaneous tissue disorders	Eczema	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	2	15.4	4.3	42.2	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0

Skin and subcutaneous tissue disorders	Rash	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR2AE_SE_20FEB2023_40703.xls
17AUG2023 14:00

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Eye disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Cystoid macular oedema	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Constipation	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Gastroenteritis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastroenteritis norovirus	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Urinary tract infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Femur fracture	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR35AE_SE_20FEB2023_40703.xls
17AUG2023 14:03

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Eye disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Eye disorders	Cystoid macular oedema	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Gastrointestinal disorders	Constipation	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	1	7.7	1.4	33.3	5	100.0	1	20.0	3.6	62.4	26	100.0	3	11.5	4.0	29.0
Infections and infestations	Gastroenteritis	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Gastroenteritis norovirus	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	1	7.7	1.4	33.3	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Infections and infestations	Urinary tract infection	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	1	20.0	3.6	62.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications		n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9
Injury, poisoning and procedural complications	Femur fracture	n/a	8	100.0	1	12.5	2.2	47.1	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	1	3.8	0.7	18.9

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR3AE_SE_20FEB2023_40703.xls
17AUG2023 14:04

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR4AE_SE_20FEB2023_40703.xls
17AUG2023 14:06

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death)
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_soc_WGR5AE_SE_20FEB2023_40703.xls
17AUG2023 14:06

POPULATION: Safety-Evaluable Population
ENDPOINT: Case of an elevated ALT or AST in combination with either an elevated Bilirubin or clinical jaundice, as defined in protocol
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_ALTAST_SE_20FEB2023_40703.xls
17AUG2023 12:48

POPULATION: Safety-Evaluable Population
ENDPOINT: Suspected transmission of an infectious agent by the study drug
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_INFAG_SE_20FEB2023_40703.xls
17AUG2023 12:52

POPULATION: Safety-Evaluable Population
ENDPOINT: Serious case of an elevated ALT or AST in combination with either an elevated Bilirubin or clinical jaundice, as defined in protocol
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_SALTAST_SE_20FEB2023_40703.xls
17AUG2023 12:51

POPULATION: Safety-Evaluable Population
ENDPOINT: Serious suspected transmission of an infectious agent by the study drug
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_SINFAG_SE_20FEB2023_40703.xls
17AUG2023 12:55

POPULATION: Safety-Evaluable Population
ENDPOINT: Case of an elevated ALT or AST in combination with either an elevated Bilirubin or clinical jaundice, as defined in protocol Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AESIs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WG35ALTAST_SE_20FEB2023_40703.xls
17AUG2023 12:49

POPULATION: Safety-Evaluable Population
ENDPOINT: Suspected transmission of an infectious agent by the study drug Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=8)						Patients with 3 SMN2 copies (N=13)						Patients with >=4 SMN2 copies (N=5)						All Patients (N=26)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	8	100.0	0	0.0	0.0	32.4	13	100.0	0	0.0	0.0	22.8	5	100.0	0	0.0	0.0	43.4	26	100.0	0	0.0	0.0	12.9

Only AESIs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_raw_WG35INFAG_SE_20FEB2023_40703.xls
17AUG2023 12:53

POPULATION: Safety-Evaluable Population
ENDPOINT: All patients
MODEL: --
STUDY: BN40703
Outcome and Causality of Adverse Events

	Patients with 2 SMN2 copies (N=8)																	
	Total	RECOVERED/RESOLVED		RECOVERED/RESOLVED WITH SEQUELAE		NOT RECOVERED/NOT RESOLVED		FATAL		RECOVERING/RESOLVING		UNKNOWN		MISSING		Causality = Yes	
Endpoint Grade	n	n	%	n	%	n	%	n	%	n	%	n	%	n	%	n	%
Any SAEs																	
All	5	5	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
Grade 1	2	2	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
Grade 3	3	3	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0

Clinical cut-off: 20FEB2023

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_resolved.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_CSRFinal/prod/output/t_ae_resolved_aerel_sae_SE_20FEB2023_40703.xls
17AUG2023 11:57

Patients with 3 SMN2 copies (N=13)																	
Total	RECOVERED/RESOLVED		RECOVERED/RESOLVED WITH SEQUELAE		NOT RECOVERED/NOT RESOLVED		FATAL		RECOVERING/RESOLVING		UNKNOWN		MISSING		Causality = Yes	
n	n	%	n	%	n	%	n	%	n	%	n	%	n	%	n	%
0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0

Patients with >=4 SMN2 copies (N=5)																	
Total	RECOVERED/RESOLVED		RECOVERED/RESOLVED WITH SEQUELAE		NOT RECOVERED/NOT RESOLVED		FATAL		RECOVERING/RESOLVING		UNKNOWN		MISSING		Causality = Yes	
n	n	%	n	%	n	%	n	%	n	%	n	%	n	%	n	%
3	3	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
2	2	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
1	1	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0

POPULATION: Intent-to-Treat Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Demographics and Baseline Characteristics

	Patients with 2 SMN2 copies (N=7)	Patients with 3 SMN2 copies (N=7)	Patients with >=4 SMN2 copies (N=4)	All Patients (N=18)
Age at Enrollment (Days)				
n	7	7	4	18
Mean (SD)	22.86 (5.37)	29.57 (7.59)	31.25 (6.99)	27.33 (7.28)
Median	23	30	32	25.5
IQR	21.0 - 24.0	21.0 - 36.0	25.5 - 37.0	21.0 - 36.0
Min - Max	15.0 - 33.0	19.0 - 38.0	23.0 - 38.0	15.0 - 38.0
Sex				
n	7	7	4	18
Male	4 (57.1%)	3 (42.9%)	1 (25.0%)	8 (44.4%)
Female	3 (42.9%)	4 (57.1%)	3 (75.0%)	10 (55.6%)
Race				
n	7	7	4	18
Asian	0	0	2 (50.0%)	2 (11.1%)
White	7 (100%)	6 (85.7%)	2 (50.0%)	15 (83.3%)
Unknown	0	1 (14.3%)	0	1 (5.6%)
Ethnicity				
n	7	7	4	18
Hispanic or Latino	3 (42.9%)	0	0	3 (16.7%)
Not Hispanic or Latino	4 (57.1%)	6 (85.7%)	4 (100%)	14 (77.8%)
Not Stated	0	1 (14.3%)	0	1 (5.6%)
Region				
n	7	7	4	18
Europe	2 (28.6%)	2 (28.6%)	2 (50.0%)	6 (33.3%)
Rest of the World	5 (71.4%)	4 (57.1%)	2 (50.0%)	11 (61.1%)
US	0	1 (14.3%)	0	1 (5.6%)
SMA Identification Method				
n	7	7	4	18
Family History	3 (42.9%)	1 (14.3%)	0	4 (22.2%)
Newborn Screening	4 (57.1%)	5 (71.4%)	4 (100%)	13 (72.2%)
Other	0	1 (14.3%)	0	1 (5.6%)

CHOP-INTEND Score				
n	7	7	4	18
Mean (SD)	44.43 (6.95)	52.86 (5.61)	47.75 (3.30)	48.44 (6.70)
Median	45	56	48	49
IQR	36.0 - 52.0	46.0 - 57.0	45.0 - 50.5	44.0 - 53.0
Min - Max	35.0 - 52.0	44.0 - 58.0	44.0 - 51.0	35.0 - 58.0
HINE-2 Score				
n	7	7	4	18
Mean (SD)	2.00 (1.53)	3.29 (1.50)	1.75 (1.50)	2.44 (1.58)
Median	2	3	1	2
IQR	1.0 - 4.0	2.0 - 4.0	1.0 - 2.5	1.0 - 4.0
Min - Max	0.0 - 4.0	2.0 - 6.0	1.0 - 4.0	0.0 - 6.0
CMAP Negative Peak Amplitude (mV)				
n	7	7	4	18
Mean (SD)	2.02 (1.26)	4.41 (1.67)	4.63 (1.39)	3.53 (1.85)
Median	2.4	4.3	4.15	3.6
IQR	0.6 - 3.0	3.2 - 6.4	3.7 - 5.6	2.4 - 4.6
Min - Max	0.5 - 3.8	2.1 - 6.7	3.6 - 6.6	0.5 - 6.7
n	7	7	4	18
<= 1mV	2 (28.6%)	0	0	2 (11.1%)
> 1mV	5 (71.4%)	7 (100%)	4 (100%)	16 (88.9%)
Current Level of Respiratory Support				
No Pulmonary Care	7 (100%)	7 (100%)	4 (100%)	18 (100%)
BiPAP Support < 16 Hours Per Day	0	0	0	0
BiPAP Support >= 16 Hours Per Day	0	0	0	0
Cough Assist - Used Daily for Therapy, Not Illness Related	0	0	0	0
Cough Assist - Used With An Illness	0	0	0	0
Ventilation Provided Prophylactically				
Awake Assisted Ventilation	0	0	0	0
Night-time Assisted Ventilation	0	0	0	0
Nap-time Assisted Ventilation	0	0	0	0
>16h Assisted Ventilation	0	0	0	0
Airway Clearance Through Cough Assistance	0	0	0	0
BiPAP Support >=16 Hours per Day for >21 Consecutive Days				
Yes	0	0	0	0
No	7 (100%)	7 (100%)	4 (100%)	18 (100%)
Intubation for >21 Consecutive Days				
Yes	0	0	0	0
No	7 (100%)	7 (100%)	4 (100%)	18 (100%)

Able to Swallow				
n	7	7	4	18
Yes	7 (100%)	7 (100%)	4 (100%)	18 (100%)
No	0	0	0	0
Missing	0	0	0	0
Able to Swallow Solid Food				
n	7	7	4	18
Yes	0	0	0	0
No	6 (85.7%)	7 (100%)	3 (75.0%)	16 (88.9%)
Missing	1 (14.3%)	0	1 (25.0%)	2 (11.1%)

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_dm.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_dm_IT_01JUL2021_40703.xls
18MAY2022 17:06

POPULATION: Intent-to-Treat Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Patient Disposition

	Patients with 2 SMN2 copies (N=7)	Patients with 3 SMN2 copies (N=7)	Patients with >=4 SMN2 copies (N=4)	All Patients (N=18)
Enrolled	7 (100%)	7 (100%)	4 (100%)	18 (100%)
Ongoing	7 (100%)	7 (100%)	4 (100%)	18 (100%)
Completed 12 months of treatment	4 (57.1%)	2 (28.6%)	1 (25.0%)	7 (38.9%)
Discontinued between 0 and 12 months of treatment	0	0	0	0
Entered extension phase	0	0	0	0
Discontinued from extension phase	0	0	0	0
Entered safety follow-up	0	0	0	0
Discontinued from safety follow-up	0	0	0	0

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ds.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ds_IT_01JUL2021_40703.xls
15FEB2022 9:07

POPULATION: Safety-Evaluable Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Duration of Follow-up for Safety

	Patients with 2 SMN2 copies (N=7)	Patients with 3 SMN2 copies (N=7)	Patients with >=4 SMN2 copies (N=4)	All Patients (N=18)
Follow-up Duration (days)				
n	7	7	4	18
Median	373	295	127.5	277

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_fu.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_fu_SE_01JUL2021_40703.xls
15FEB2022 9:33

POPULATION: Intent-to-Treat Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Duration of Follow-up for Efficacy

	Patients with 2 SMN2 copies (N=7)	Patients with 3 SMN2 copies (N=7)	Patients with >=4 SMN2 copies (N=4)	All Patients (N=18)
Follow-up Duration (days)				
n	7	7	4	18
Median	373	295	127.5	277

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_fu2.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_fu2_IT_01JUL2021_40703.xls
15FEB2022 9:32

POPULATION: Safety-Evaluable Population
ENDPOINT: --
MODEL: descriptive
STUDY: BN40703
Number of Patients who Died including Primary Reason

Null Report: No observations met the reporting criteria for inclusion in this output.

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_dd.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_dd_SE_01JUL2021_40703.xls
15FEB2022 9:09

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death (from enrollment)
MODEL: Unstratified Analysis
STUDY: BN40703
Time to Event Analysis (Efficacy)

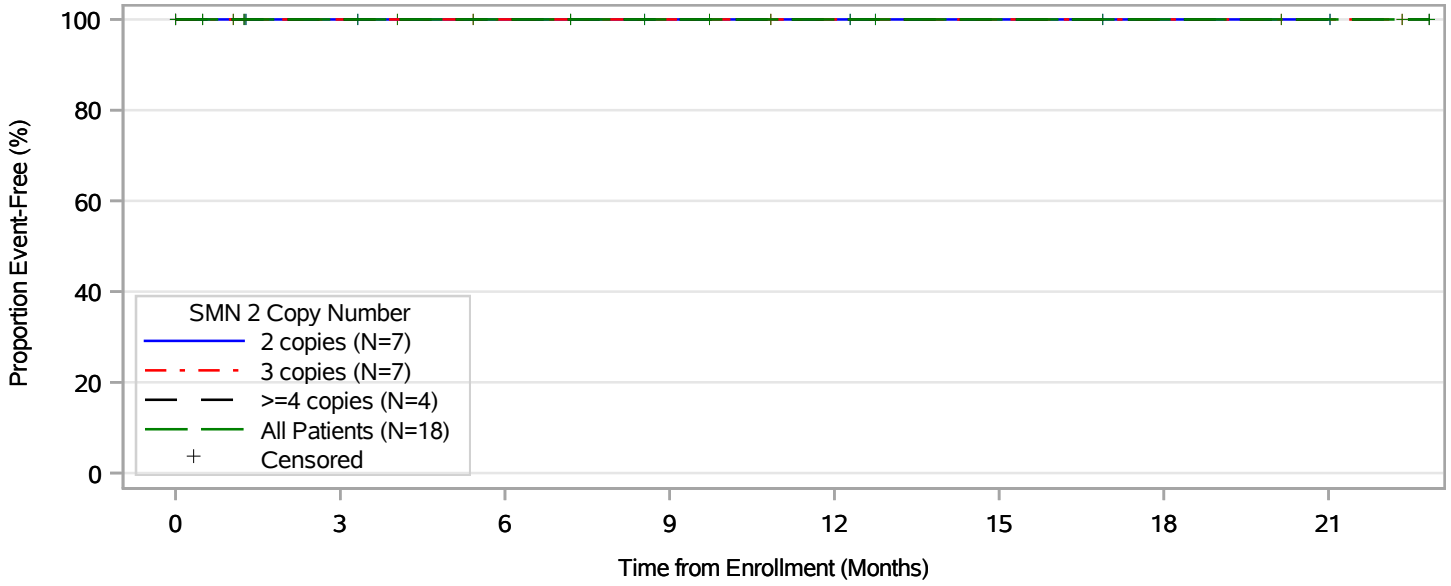
		Patients with 2 SMN2 copies (N=7)												Patients with 3 SMN2 copies (N=7)											
		Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
Name	Level	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
All	n/a	7	100.0	0	0	7	100.0	NE	NE	NE	NE	NE	NE	7	100.0	0	0	7	100.0	NE	NE	NE	NE	NE	NE

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_tte.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_tte_DTH_IT_01JUL2021_40703.xls
28JAN2022 15:46

Patients with >=4 SMN2 copies (N=4)												All Patients (N=18)											
Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
4	100.0	0	0	4	100.0	NE	NE	NE	NE	NE	NE	18	100.0	0	0	18	100.0	NE	NE	NE	NE	NE	NE

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death (from enrollment)
STUDY: BN40703



Patients at risk								
2 copies (N=7)	7	6	5	4	4	2	1	1
3 copies (N=7)	7	6	4	4	2	2	2	1
>=4 copies (N=4)	4	2	2	1	1	1	1	1
All Patients (N=18)	18	14	11	9	7	5	4	3
Patients censored								
2 copies (N=7)	0	1	2	3	3	5	6	6
3 copies (N=7)	0	1	3	3	5	5	5	6
>=4 copies (N=4)	0	2	2	3	3	3	3	3
All Patients (N=18)	0	4	7	9	11	13	14	15

Clinical cut-off: 01JUL2021

Program: ..al_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/g_km.sas
Output: ..03/data_analysis/ACE_eSub_sNDA_analysis/prod/output/g_km_DTH_IT_01JUL2021_40703.pdf
28JAN2022 15:50

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death or Permanent Ventilation (from enrollment)
MODEL: Unstratified Analysis
STUDY: BN40703
Time to Event Analysis (Efficacy)

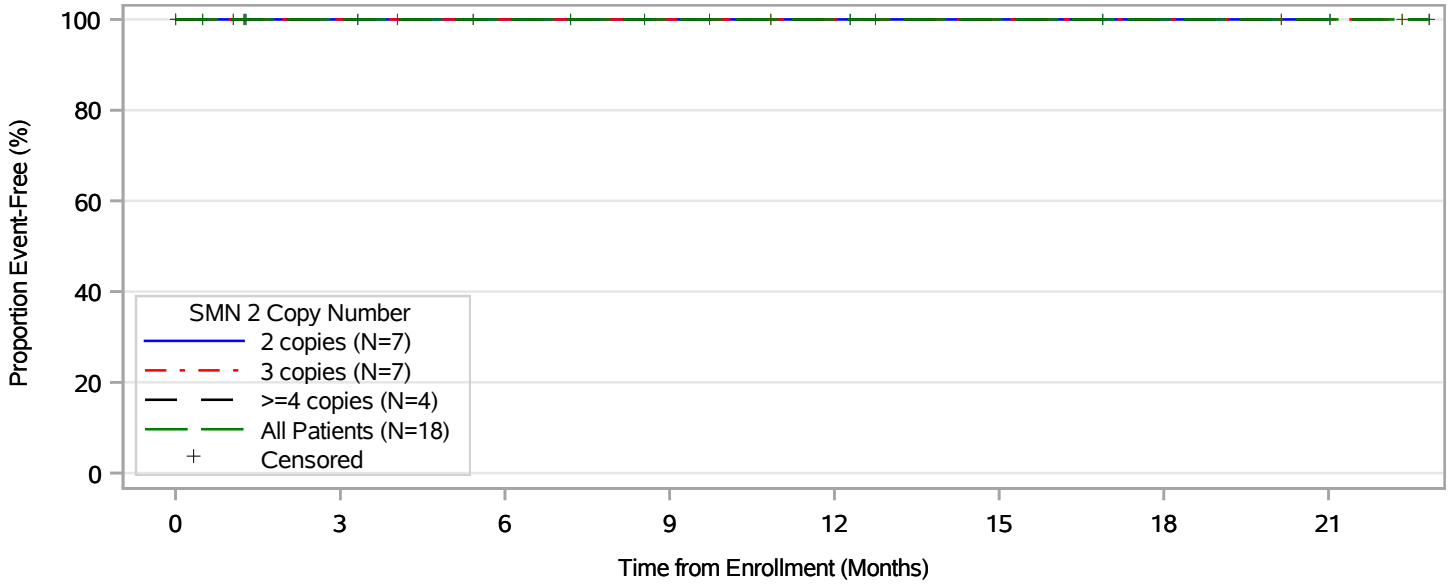
		Patients with 2 SMN2 copies (N=7)												Patients with 3 SMN2 copies (N=7)											
		Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
Name	Level	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
All	n/a	7	100.0	0	0	7	100.0	NE	NE	NE	NE	NE	NE	7	100.0	0	0	7	100.0	NE	NE	NE	NE	NE	NE

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_tte.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_tte_PVDTH_IT_01JUL2021_40703.xls
28JAN2022 15:49

Patients with >=4 SMN2 copies (N=4)												All Patients (N=18)											
Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
4	100.0	0	0	4	100.0	NE	NE	NE	NE	NE	NE	18	100.0	0	0	18	100.0	NE	NE	NE	NE	NE	NE

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Death or Permanent Ventilation (from enrollment)
STUDY: BN40703



Patients at risk								
2 copies (N=7)	7	6	5	4	4	2	1	1
3 copies (N=7)	7	6	4	4	2	2	2	1
>=4 copies (N=4)	4	2	2	1	1	1	1	1
All Patients (N=18)	18	14	11	9	7	5	4	3
Patients censored								
2 copies (N=7)	0	1	2	3	3	5	6	6
3 copies (N=7)	0	1	3	3	5	5	5	6
>=4 copies (N=4)	0	2	2	3	3	3	3	3
All Patients (N=18)	0	4	7	9	11	13	14	15

Clinical cut-off: 01JUL2021

Program: ..al_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/g_km.sas
Output: ../data_analysis/ACE_eSub_sNDA_analysis/prod/output/g_km_PVDTH_IT_01JUL2021_40703.pdf
28JAN2022 15:52

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Permanent Ventilation (from enrollment)
MODEL: Unstratified Analysis
STUDY: BN40703
Time to Event Analysis (Efficacy)

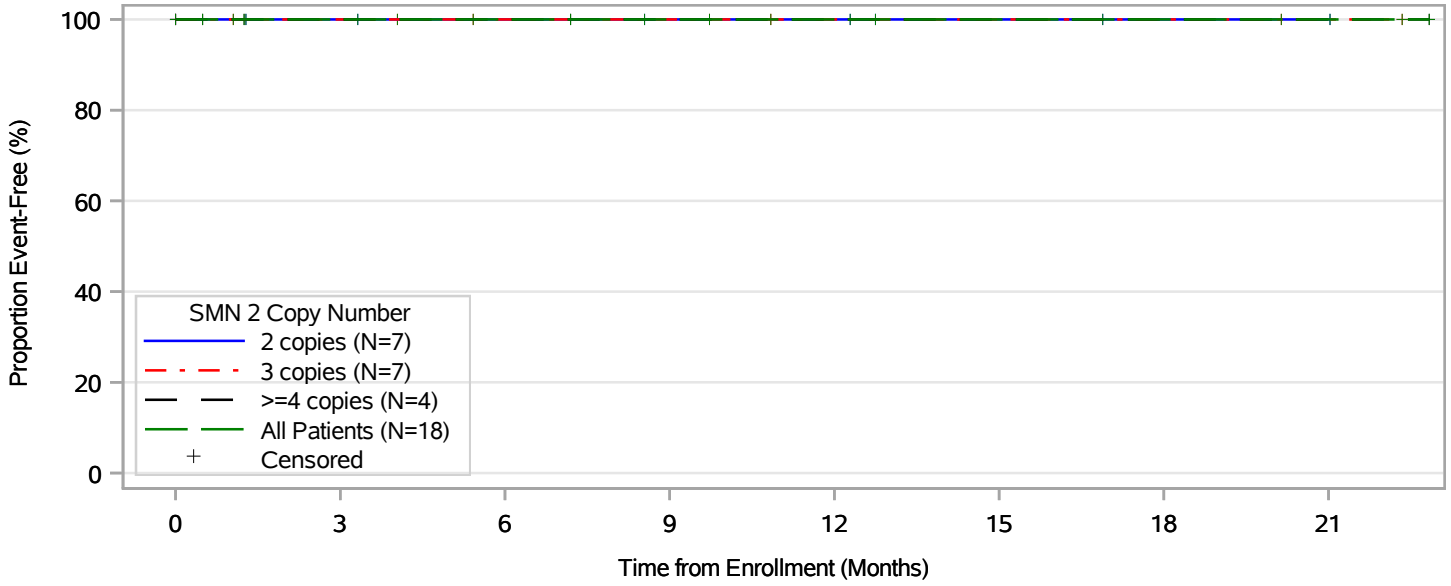
		Patients with 2 SMN2 copies (N=7)												Patients with 3 SMN2 copies (N=7)											
		Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
Name	Level	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
All	n/a	7	100.0	0	0	7	100.0	NE	NE	NE	NE	NE	NE	7	100.0	0	0	7	100.0	NE	NE	NE	NE	NE	NE

Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_tte.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_tte_PV_IT_01JUL2021_40703.xls
28JAN2022 15:47

Patients with >=4 SMN2 copies (N=4)												All Patients (N=18)											
Patients		Patients with Event		Censored		Time to event						Patients		Patients with Event		Censored		Time to event					
n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median	n	%	n	%	n	%	Q1 (months)	95% Lower CL for Q1	95% Upper CL for Q1	Median (months)	95% Lower CL for Median	95% Upper CL for Median
4	100.0	0	0	4	100.0	NE	NE	NE	NE	NE	NE	18	100.0	0	0	18	100.0	NE	NE	NE	NE	NE	NE

POPULATION: Intent-to-Treat Population
ENDPOINT: Time to Permanent Ventilation (from enrollment)
STUDY: BN40703



Patients at risk								
2 copies (N=7)	7	6	5	4	4	2	1	1
3 copies (N=7)	7	6	4	4	2	2	2	1
>=4 copies (N=4)	4	2	2	1	1	1	1	1
All Patients (N=18)	18	14	11	9	7	5	4	3
Patients censored								
2 copies (N=7)	0	1	2	3	3	5	6	6
3 copies (N=7)	0	1	3	3	5	5	5	6
>=4 copies (N=4)	0	2	2	3	3	3	3	3
All Patients (N=18)	0	4	7	9	11	13	14	15

Clinical cut-off: 01JUL2021

Program: ..al_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/g_km.sas
Output: ..703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/g_km_PV_IT_01JUL2021_40703.pdf
28JAN2022 15:51

POPULATION: Intent-to-Treat Population
ENDPOINT: CHOP-INTEND total score
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	7	100.0	7	100.0	44.43	6.95	7	100.0	7	100.0	52.86	5.61	4	100.0	4	100.0	47.75	3.30	18	100.0	18	100.0	48.44	6.70
Week 4	n/a	7	100.0	7	100.0	44.29	9.78	7	100.0	7	100.0	57.00	5.60	3	75.0	3	100.0	53.33	1.53	17	94.4	17	100.0	51.12	9.18
Week 8	n/a	6	85.7	5	83.3	51.80	6.87	6	85.7	6	100.0	60.50	1.22	2	50.0	2	100.0	53.00	2.83	14	77.8	13	92.9	56.00	6.00
Week 16	n/a	6	85.7	5	83.3	57.00	8.60	6	85.7	6	100.0	61.83	2.04	2	50.0	2	100.0	61.00	1.41	14	77.8	13	92.9	59.85	5.67
Week 28	n/a	5	71.4	5	100.0	61.00	2.55	5	71.4	3	60.0	63.33	1.15	2	50.0	1	50.0	62.00	NE	12	66.7	9	75.0	61.89	2.20
Week 40	n/a	5	71.4	4	80.0	61.00	4.76	4	57.1	4	100.0	63.50	1.00	1	25.0	1	100.0	64.00	NE	10	55.6	9	90.0	62.44	3.28
Week 52	n/a	4	57.1	4	100.0	59.00	5.83	3	42.9	2	66.7	64.00	0.00	1	25.0	1	100.0	63.00	NE	8	44.4	7	87.5	61.00	4.83
Week 64	n/a	2	28.6	1	50.0	64.00	NE	2	28.6	2	100.0	64.00	0.00	1	25.0	1	100.0	64.00	NE	5	27.8	4	80.0	64.00	0.00
Week 78	n/a	2	28.6	0	NE	NE	NE	2	28.6	0	NE	NE	NE	1	25.0	0	NE	NE	NE	5	27.8	0	NE	NE	NE
Week 92	n/a	1	14.3	0	NE	NE	NE	2	28.6	0	NE	NE	NE	1	25.0	0	NE	NE	NE	4	22.2	0	NE	NE	NE

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - absolute values
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_mean_CHOP_IT_01JUL2021_40703.xls
30AUG2023 10:17

POPULATION: Intent-to-Treat Population
ENDPOINT: Change from Baseline in CHOP-INTEND total score up to Month 12
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	7	100.0	7	100.0	NE	NE	7	100.0	7	100.0	NE	NE	4	100.0	4	100.0	NE	NE	18	100.0	18	100.0	NE	NE
Week 4	n/a	7	100.0	7	100.0	-0.14	4.91	7	100.0	7	100.0	4.14	5.67	3	75.0	3	100.0	5.00	5.20	17	94.4	17	100.0	2.53	5.47
Week 8	n/a	6	85.7	5	83.3	5.80	3.27	6	85.7	6	100.0	6.17	3.92	2	50.0	2	100.0	6.00	7.07	14	77.8	13	92.9	6.00	3.76
Week 16	n/a	6	85.7	5	83.3	10.60	3.91	6	85.7	6	100.0	7.50	4.59	2	50.0	2	100.0	14.00	5.66	14	77.8	13	92.9	9.69	4.73
Week 28	n/a	5	71.4	5	100.0	14.60	6.58	5	71.4	3	60.0	7.33	1.53	2	50.0	1	50.0	18.00	NE	12	66.7	9	75.0	12.56	6.23
Week 40	n/a	5	71.4	4	80.0	16.00	4.24	4	57.1	4	100.0	10.00	5.94	1	25.0	1	100.0	20.00	NE	10	55.6	9	90.0	13.78	5.87
Week 52	n/a	4	57.1	4	100.0	14.00	8.83	3	42.9	2	66.7	14.50	4.95	1	25.0	1	100.0	19.00	NE	8	44.4	7	87.5	14.86	6.82

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - change from baseline
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_mean_chg_CHOP_IT_01JUL2021_40703.xls
01FEB2022 16:32

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of patients who achieve a score of 40 or higher in the CHOP-INTEND at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	4	57.1	4	100.0	51.0	100.0	2	28.6	2	100.0	34.2	100.0	1	25.0	1	100.0	20.7	100.0	7	38.9	7	100.0	64.6	100.0

95% CI based on Wilson Scores.
Only patients reaching 52 weeks of treatment are included in the analysis.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_resp_CHOP40_IT_01JUL2021_40703.xls
28JAN2022 15:53

POPULATION: Intent-to-Treat Population
ENDPOINT: HINE-2 total score
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	7	100.0	7	100.0	2.00	1.53	7	100.0	7	100.0	3.29	1.50	4	100.0	4	100.0	1.75	1.50	18	100.0	18	100.0	2.44	1.58
Week 4	n/a	7	100.0	7	100.0	2.00	1.53	7	100.0	7	100.0	4.86	2.27	3	75.0	3	100.0	4.00	1.00	17	94.4	17	100.0	3.53	2.18
Week 8	n/a	6	85.7	6	100.0	2.83	1.94	6	85.7	6	100.0	6.33	2.66	2	50.0	2	100.0	5.00	2.83	14	77.8	14	100.0	4.64	2.76
Week 16	n/a	6	85.7	5	83.3	5.80	1.92	6	85.7	6	100.0	10.17	3.43	2	50.0	2	100.0	8.00	2.83	14	77.8	13	92.9	8.15	3.34
Week 28	n/a	5	71.4	5	100.0	12.40	4.04	5	71.4	3	60.0	18.33	0.58	2	50.0	2	100.0	18.00	2.83	12	66.7	10	83.3	15.30	4.19
Week 40	n/a	5	71.4	4	80.0	16.25	6.24	4	57.1	4	100.0	22.50	1.00	1	25.0	1	100.0	20.00	NE	10	55.6	9	90.0	19.44	4.98
Week 52	n/a	4	57.1	4	100.0	20.25	5.62	3	42.9	2	66.7	25.50	0.71	1	25.0	1	100.0	22.00	NE	8	44.4	7	87.5	22.00	4.69
Week 64	n/a	2	28.6	2	100.0	24.50	2.12	2	28.6	2	100.0	26.00	0.00	1	25.0	1	100.0	26.00	NE	5	27.8	5	100.0	25.40	1.34
Week 78	n/a	2	28.6	1	50.0	25.00	NE	2	28.6	2	100.0	25.50	0.71	1	25.0	1	100.0	26.00	NE	5	27.8	4	80.0	25.50	0.58
Week 92	n/a	1	14.3	0	NE	NE	NE	2	28.6	0	NE	NE	NE	1	25.0	1	100.0	26.00	NE	4	22.2	1	25.0	26.00	NE

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - absolute values
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_mean_HINE_IT_01JUL2021_40703.xls
30AUG2023 10:18

POPULATION: Intent-to-Treat Population
ENDPOINT: Change from Baseline in HINE-2 total score up to Month 12
MODEL: --
STUDY: BN40703
Compliance and Mean

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients				Statistics		Patients				Statistics		Patients				Statistics		Patients				Statistics	
Name Visit	Level	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)	in study[1]	%	with value[1]	%	Mean[2]	SD(Mean)
All																									
BASELINE	n/a	7	100.0	7	100.0	NE	NE	7	100.0	7	100.0	NE	NE	4	100.0	4	100.0	NE	NE	18	100.0	18	100.0	NE	NE
Week 4	n/a	7	100.0	7	100.0	0.00	1.91	7	100.0	7	100.0	1.57	1.13	3	75.0	3	100.0	2.00	2.00	17	94.4	17	100.0	1.00	1.77
Week 8	n/a	6	85.7	6	100.0	0.83	1.17	6	85.7	6	100.0	2.83	2.32	2	50.0	2	100.0	4.00	2.83	14	77.8	14	100.0	2.14	2.18
Week 16	n/a	6	85.7	5	83.3	4.20	2.17	6	85.7	6	100.0	6.67	3.08	2	50.0	2	100.0	7.00	2.83	14	77.8	13	92.9	5.77	2.80
Week 28	n/a	5	71.4	5	100.0	10.80	4.09	5	71.4	3	60.0	15.33	1.15	2	50.0	2	100.0	17.00	2.83	12	66.7	10	83.3	13.40	4.06
Week 40	n/a	5	71.4	4	80.0	14.75	6.24	4	57.1	4	100.0	19.25	0.96	1	25.0	1	100.0	19.00	NE	10	55.6	9	90.0	17.22	4.52
Week 52	n/a	4	57.1	4	100.0	18.75	5.68	3	42.9	2	66.7	22.00	0.00	1	25.0	1	100.0	21.00	NE	8	44.4	7	87.5	20.00	4.32

[1] in study: number of subjects in study at respective visit; % based on baseline
with value: number of subjects in study and with value at respective visit - used for the calculation of the mean and SD; % based on patients in study at respective visit
[2] mean: descriptive statistics - change from baseline
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_mean.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_mean_chg_HINE_IT_01JUL2021_40703.xls
01FEB2022 16:32

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants who achieve the attainment levels of the motor milestones assessed in HINE-2 at Month 12
MODEL: --
STUDY: BN40703

Name	Level		Patients with 2 SMN2 copies (N=7)	Patients with 3 SMN2 copies (N=7)	Patients with >=4 SMN2 copies (N=4)	All Patients (N=18)
Head Control						
All	n/a	n	4	2	1	7
		Wobbles	1 (25.0%)	0	0	1 (14.3%)
		All the time maintained upright	3 (75.0%)	2 (100%)	1 (100%)	6 (85.7%)
Sitting						
All	n/a	n	4	2	1	7
		Stable sit	1 (25.0%)	0	0	1 (14.3%)
		Pivots (rotates)	3 (75.0%)	2 (100%)	1 (100%)	6 (85.7%)
Voluntary Grasp						
All	n/a	n	4	2	1	7
		Index finger and thumb but immature grasp	1 (25.0%)	1 (50.0%)	0	2 (28.6%)
		Pincer grasp	3 (75.0%)	1 (50.0%)	1 (100%)	5 (71.4%)
Ability to Kick (in Supine)						
All	n/a	n	4	2	1	7
		Touches toes	4 (100%)	2 (100%)	1 (100%)	7 (100%)
Rolling						
All	n/a	n	4	2	1	7
		Prone to supine	1 (25.0%)	0	0	1 (14.3%)
		Supine to prone	3 (75.0%)	2 (100%)	1 (100%)	6 (85.7%)
Crawling						
All	n/a	n	4	2	1	7
		On elbow	1 (25.0%)	0	0	1 (14.3%)
		Crawling flat on abdomen	1 (25.0%)	0	0	1 (14.3%)
		Crawling on hands and knees	2 (50.0%)	2 (100%)	1 (100%)	5 (71.4%)
Standing						
All	n/a	n	4	2	1	7
		Does not support weight	2 (50.0%)	0	0	2 (28.6%)
		Stands with support	1 (25.0%)	0	1 (100%)	2 (28.6%)

		Stands unaided	1 (25.0%)	2 (100%)	0	3 (42.9%)
Walking						
All	n/a	n	3	2	1	6
		Bouncing	1 (33.3%)	0	0	1 (16.7%)
		Cannot Test	1 (33.3%)	0	1 (100%)	2 (33.3%)
		Walking Independently	1 (33.3%)	2 (100%)	0	3 (50.0%)

Only patients reaching 52 weeks of treatment are included in the analysis.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_hine.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_hine_IT_01JUL2021_40703.xls
18MAR2022 8:42

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of motor milestone responders (HINE-2) at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	4	57.1	4	100.0	51.0	100.0	2	28.6	2	100.0	34.2	100.0	1	25.0	1	100.0	20.7	100.0	7	38.9	7	100.0	64.6	100.0

95% CI based on Wilson Scores.
Only patients reaching 52 weeks of treatment are included in the analysis.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_resp_MMR_IT_01JUL2021_40703.xls
31JAN2022 16:23

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to swallow at Baseline
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	7	100.0	64.6	100.0	7	100.0	7	100.0	64.6	100.0	4	100.0	4	100.0	51.0	100.0	18	100.0	18	100.0	82.4	100.0

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_resp_bl_ASLW_IT_01JUL2021_40703.xls
07SEP2023 11:23

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to swallow at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	4	57.1	4	100.0	51.0	100.0	2	28.6	2	100.0	34.2	100.0	1	25.0	1	100.0	20.7	100.0	7	38.9	7	100.0	64.6	100.0

95% CI based on Wilson Scores.
Only patients reaching 52 weeks of treatment are included in the analysis.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_resp_ASLW_IT_01JUL2021_40703.xls
31JAN2022 16:29

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to feed orally at Baseline
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	7	100.0	64.6	100.0	7	100.0	7	100.0	64.6	100.0	4	100.0	4	100.0	51.0	100.0	18	100.0	18	100.0	82.4	100.0

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_resp_bl_FEED_IT_01JUL2021_40703.xls
07SEP2023 11:24

POPULATION: Intent-to-Treat Population
ENDPOINT: Proportion of infants with the ability to feed orally at Month 12
MODEL: Unstratified Analysis
STUDY: BN40703
Dichotomous Analysis (Efficacy)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	4	57.1	4	100.0	51.0	100.0	2	28.6	2	100.0	34.2	100.0	1	25.0	1	100.0	20.7	100.0	7	38.9	7	100.0	64.6	100.0

95% CI based on Wilson Scores.
Only patients reaching 52 weeks of treatment are included in the analysis.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_resp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_resp_FEED_IT_01JUL2021_40703.xls
31JAN2022 16:29

POPULATION: Intent-to-Treat Population
ENDPOINT: Number of Hospitalizations (for any reason) per Patient-Year at Month 12.
MODEL: --
STUDY: BN40703

			Patients with 2 SMN2 copies (N=7)	Patients with 3 SMN2 copies (N=7)	Patients with >=4 SMN2 copies (N=4)	All Patients (N=18)
Name	Level		Statistics	Statistics	Statistics	Statistics
All	n/a	Total patient-years at risk	5.2	4.4	1.7	11.4
		Number of hospitalizations	0	0	0	0
		Number of hospitalizations per patient-year	NE	NE	NE	NE
		95% CI	NE	NE	NE	NE

Total patient-years at risk is the sum over all patients of the time intervals (in years) from the start of study treatment to the earliest date of study withdrawal or completion of 12 months of treatment (Month 12 visit).
Includes all hospital admissions observed by Month 12 which span at least two days.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_hosp.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_hosp_IT_01JUL2021_40703.xls
28JAN2022 15:58

POPULATION: Intent-to-Treat Population
ENDPOINT: Number of Nights Admitted to Hospital per Infant at Month 12.
MODEL: --
STUDY: BN40703

Null Report: No observations met the reporting criteria for inclusion in this output.

Includes all hospital admissions observed by Month 12 which span at least two days.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_eff_hosp_nights.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_eff_hosp_nights_IT_01JUL2021_40703.xls
28JAN2022 15:58

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	5	71.4	35.9	91.8	7	100.0	6	85.7	48.7	97.4	4	100.0	3	75.0	30.1	95.4	18	100.0	14	77.8	54.8	91.0

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_AE_SE_01JUL2021_40703.xls
28JAN2022 14:46

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	4	57.1	25.0	84.2	7	100.0	6	85.7	48.7	97.4	4	100.0	3	75.0	30.1	95.4	18	100.0	13	72.2	49.1	87.5

95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_AE_EXCL_SE_01JUL2021_40703.xls
07APR2022 17:17

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	5	71.4	35.9	91.8	7	100.0	6	85.7	48.7	97.4	4	100.0	3	75.0	30.1	95.4	18	100.0	14	77.8	54.8	91.0

95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_AE_EXCLN_SE_01JUL2021_40703.xls
07APR2022 16:22

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_SAE_SE_01JUL2021_40703.xls
28JAN2022 14:58

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_SAE_EXCL_SE_01JUL2021_40703.xls
07APR2022 17:27

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_SAE_EXCLN_SE_01JUL2021_40703.xls
07APR2022 16:31

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs leading to any Study Treatment Discontinuation
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WDSTAE_SE_01JUL2021_40703.xls
28JAN2022 14:59

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1/2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	4	57.1	25.0	84.2	7	100.0	5	71.4	35.9	91.8	4	100.0	3	75.0	30.1	95.4	18	100.0	12	66.7	43.7	83.7

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR12AE_SE_01JUL2021_40703.xls
28JAN2022 14:51

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	3	42.9	15.8	75.0	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	7	38.9	20.3	61.4

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR1AE_SE_01JUL2021_40703.xls
28JAN2022 14:48

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	2	28.6	8.2	64.1	4	100.0	2	50.0	15.0	85.0	18	100.0	5	27.8	12.5	50.9

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR2AE_SE_01JUL2021_40703.xls
28JAN2022 14:49

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR35AE_SE_01JUL2021_40703.xls
28JAN2022 14:52

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5 Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR35AE_EXCL_SE_01JUL2021_40703.xls
07APR2022 17:22

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5 Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR35AE_EXCLN_SE_01JUL2021_40703.xls
07APR2022 16:27

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR3AE_SE_01JUL2021_40703.xls
28JAN2022 14:53

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3 Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR3AE_EXCL_SE_01JUL2021_40703.xls
07APR2022 17:23

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3 Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR3AE_EXCLN_SE_01JUL2021_40703.xls
07APR2022 16:28

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR4AE_SE_01JUL2021_40703.xls
28JAN2022 14:55

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4 Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR4AE_EXCL_SE_01JUL2021_40703.xls
07APR2022 17:24

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4 Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR4AE_EXCLN_SE_01JUL2021_40703.xls
07APR2022 16:29

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death)
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR5AE_SE_01JUL2021_40703.xls
28JAN2022 14:56

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death) Excluding Broad Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the broad prospective-PT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR5AE_EXCL_SE_01JUL2021_40703.xls
07APR2022 17:26

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death) Excluding Narrow Basket Disease Related Adverse Events
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AEs of highest severity are counted.
95% CI based on Wilson Scores.
Disease related events are defined as those under the narrow prospective-LLT basket.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WGR5AE_EXCLN_SE_01JUL2021_40703.xls
07APR2022 16:30

POPULATION: Safety-Evaluable Population
ENDPOINT: Any AEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Eye disorders		n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Eye disorders	Cystoid macular oedema	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	5	71.4	35.9	91.8	4	100.0	2	50.0	15.0	85.0	18	100.0	9	50.0	29.0	71.0
Gastrointestinal disorders	Abdominal pain	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Gastrointestinal disorders	Constipation	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	3	16.7	5.8	39.2
Gastrointestinal disorders	Diarrhoea	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	2	50.0	15.0	85.0	18	100.0	4	22.2	9.0	45.2
Gastrointestinal disorders	Gastrointestinal pain	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Gastrooesophageal reflux disease	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Teething	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	4	57.1	25.0	84.2	4	100.0	0	0.0	0.0	49.0	18	100.0	6	33.3	16.3	56.3
Gastrointestinal disorders	Vomiting	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	4	22.2	9.0	45.2
General disorders and administration site conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	3	42.9	15.8	75.0	4	100.0	2	50.0	15.0	85.0	18	100.0	5	27.8	12.5	50.9
General disorders and administration site conditions	Malaise	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
General disorders and administration site conditions	Pyrexia	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	3	42.9	15.8	75.0	4	100.0	2	50.0	15.0	85.0	18	100.0	5	27.8	12.5	50.9
Infections and infestations		n/a	7	100.0	4	57.1	25.0	84.2	7	100.0	4	57.1	25.0	84.2	4	100.0	1	25.0	4.6	69.9	18	100.0	9	50.0	29.0	71.0
Infections and infestations	Bronchiolitis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Conjunctivitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Croup infectious	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Cytomegalovirus infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Ear infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Gastroenteritis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Gastroenteritis norovirus	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Influenza	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Nasopharyngitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Oral candidiasis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Respiratory syncytial virus infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Rhinitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Skin infection	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Varicella	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Viral infection	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	4	22.2	9.0	45.2
Infections and infestations	Vulvovaginitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Injury, poisoning and procedural complications		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Injury, poisoning and procedural complications	Accidental overdose	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Injury, poisoning and procedural complications	Contusion	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Pregnancy, puerperium and perinatal conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Pregnancy, puerperium and perinatal conditions	Umbilical granuloma	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Respiratory, thoracic and mediastinal disorders		n/a	7	100.0	3	42.9	15.8	75.0	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	7	38.9	20.3	61.4
Respiratory, thoracic and mediastinal disorders	Cough	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Respiratory, thoracic and mediastinal disorders	Nasal congestion	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	5	27.8	12.5	50.9
Respiratory, thoracic and mediastinal disorders	Rhinorrhoea	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	3	42.9	15.8	75.0	4	100.0	2	50.0	15.0	85.0	18	100.0	7	38.9	20.3	61.4
Skin and subcutaneous tissue disorders	Dermatitis contact	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Dermatitis diaper	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Eczema	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Skin and subcutaneous tissue disorders	Erythema	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Papule	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders	Rash	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Rash maculo-papular	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8

Skin and subcutaneous tissue disorders	Skin discolouration	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_AE_SE_01JUL2021_40703.xls
31JAN2022 8:34

POPULATION: Safety-Evaluable Population
ENDPOINT: Any SAEs
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_SAE_SE_01JUL2021_40703.xls
31JAN2022 8:45

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs leading to any Study Treatment Discontinuation
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WDSTAE_SE_01JUL2021_40703.xls
31JAN2022 8:46

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1/2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Gastrointestinal disorders		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	5	71.4	35.9	91.8	4	100.0	2	50.0	15.0	85.0	18	100.0	9	50.0	29.0	71.0
Gastrointestinal disorders	Abdominal pain	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Gastrointestinal disorders	Constipation	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	3	16.7	5.8	39.2
Gastrointestinal disorders	Diarrhoea	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	2	50.0	15.0	85.0	18	100.0	4	22.2	9.0	45.2
Gastrointestinal disorders	Gastrointestinal pain	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Gastrooesophageal reflux disease	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Teething	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	4	57.1	25.0	84.2	4	100.0	0	0.0	0.0	49.0	18	100.0	6	33.3	16.3	56.3
Gastrointestinal disorders	Vomiting	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	4	22.2	9.0	45.2
General disorders and administration site conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	3	42.9	15.8	75.0	4	100.0	2	50.0	15.0	85.0	18	100.0	5	27.8	12.5	50.9
General disorders and administration site conditions	Malaise	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
General disorders and administration site conditions	Pyrexia	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	3	42.9	15.8	75.0	4	100.0	2	50.0	15.0	85.0	18	100.0	5	27.8	12.5	50.9
Infections and infestations		n/a	7	100.0	4	57.1	25.0	84.2	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	8	44.4	24.6	66.3
Infections and infestations	Bronchiolitis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Conjunctivitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Croup infectious	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Cytomegalovirus infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Ear infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Gastroenteritis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Influenza	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Nasopharyngitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Oral candidiasis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Respiratory syncytial virus infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Rhinitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Skin infection	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Varicella	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Viral infection	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	4	22.2	9.0	45.2
Infections and infestations	Vulvovaginitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Injury, poisoning and procedural complications		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Injury, poisoning and procedural complications	Accidental overdose	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Injury, poisoning and procedural complications	Contusion	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Pregnancy, puerperium and perinatal conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Pregnancy, puerperium and perinatal conditions	Umbilical granuloma	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Respiratory, thoracic and mediastinal disorders		n/a	7	100.0	3	42.9	15.8	75.0	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	7	38.9	20.3	61.4
Respiratory, thoracic and mediastinal disorders	Cough	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Respiratory, thoracic and mediastinal disorders	Nasal congestion	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	5	27.8	12.5	50.9
Respiratory, thoracic and mediastinal disorders	Rhinorrhoea	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	3	42.9	15.8	75.0	4	100.0	2	50.0	15.0	85.0	18	100.0	7	38.9	20.3	61.4
Skin and subcutaneous tissue disorders	Dermatitis contact	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Dermatitis diaper	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Eczema	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Skin and subcutaneous tissue disorders	Erythema	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Papule	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders	Rash	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Rash maculo-papular	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Skin discolouration	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR12AE_SE_01JUL2021_40703.xls
31JAN2022 8:38

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 1
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Gastrointestinal disorders		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	4	57.1	25.0	84.2	4	100.0	1	25.0	4.6	69.9	18	100.0	7	38.9	20.3	61.4
Gastrointestinal disorders	Abdominal pain	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Gastrointestinal disorders	Constipation	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	3	16.7	5.8	39.2
Gastrointestinal disorders	Diarrhoea	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Gastrointestinal disorders	Gastrointestinal pain	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Gastrooesophageal reflux disease	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Teething	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	4	57.1	25.0	84.2	4	100.0	0	0.0	0.0	49.0	18	100.0	6	33.3	16.3	56.3
Gastrointestinal disorders	Vomiting	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
General disorders and administration site conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
General disorders and administration site conditions	Malaise	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
General disorders and administration site conditions	Pyrexia	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Infections and infestations		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	3	42.9	15.8	75.0	4	100.0	0	0.0	0.0	49.0	18	100.0	5	27.8	12.5	50.9
Infections and infestations	Conjunctivitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Cytomegalovirus infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Gastroenteritis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Influenza	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Oral candidiasis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Rhinitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Infections and infestations	Skin infection	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Varicella	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Viral infection	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	3	16.7	5.8	39.2
Injury, poisoning and procedural complications		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Injury, poisoning and procedural complications	Accidental overdose	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Injury, poisoning and procedural complications	Contusion	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Pregnancy, puerperium and perinatal conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Pregnancy, puerperium and perinatal conditions	Umbilical granuloma	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Respiratory, thoracic and mediastinal disorders		n/a	7	100.0	3	42.9	15.8	75.0	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	6	33.3	16.3	56.3
Respiratory, thoracic and mediastinal disorders	Cough	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Respiratory, thoracic and mediastinal disorders	Nasal congestion	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	3	42.9	15.8	75.0	4	100.0	1	25.0	4.6	69.9	18	100.0	5	27.8	12.5	50.9
Respiratory, thoracic and mediastinal disorders	Rhinorrhoea	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	4	22.2	9.0	45.2
Skin and subcutaneous tissue disorders	Dermatitis contact	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Eczema	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders	Erythema	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Papule	n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	2	11.1	3.1	32.8
Skin and subcutaneous tissue disorders	Rash maculo-papular	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Skin discolouration	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR1AE_SE_01JUL2021_40703.xls
31JAN2022 8:36

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 2
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Gastrointestinal disorders		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	1	25.0	4.6	69.9	18	100.0	2	11.1	3.1	32.8
Gastrointestinal disorders	Diarrhoea	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Gastrointestinal disorders	Vomiting	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
General disorders and administration site conditions		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	2	50.0	15.0	85.0	18	100.0	3	16.7	5.8	39.2
General disorders and administration site conditions	Pyrexia	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	2	50.0	15.0	85.0	18	100.0	3	16.7	5.8	39.2
Infections and infestations		n/a	7	100.0	2	28.6	8.2	64.1	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Infections and infestations	Bronchiolitis	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Croup infectious	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Ear infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Gastroenteritis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Nasopharyngitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Respiratory syncytial virus infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Viral infection	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Vulvovaginitis	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Injury, poisoning and procedural complications		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Injury, poisoning and procedural complications	Accidental overdose	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Respiratory, thoracic and mediastinal disorders		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Respiratory, thoracic and mediastinal disorders	Cough	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	2	28.6	8.2	64.1	4	100.0	1	25.0	4.6	69.9	18	100.0	3	16.7	5.8	39.2
Skin and subcutaneous tissue disorders	Dermatitis diaper	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Eczema	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	1	25.0	4.6	69.9	18	100.0	1	5.6	1.0	25.8
Skin and subcutaneous tissue disorders	Rash	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR2AE_SE_01JUL2021_40703.xls
31JAN2022 8:37

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Eye disorders		n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Eye disorders	Cystoid macular oedema	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Gastroenteritis norovirus	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR35AE_SE_01JUL2021_40703.xls
31JAN2022 8:39

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 3
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

All

			Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
			Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
MedDRA System Organ Class	MedDRA Preferred Term	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
Eye disorders		n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Eye disorders	Cystoid macular oedema	n/a	7	100.0	1	14.3	2.6	51.3	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations		n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8
Infections and infestations	Gastroenteritis norovirus	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	1	14.3	2.6	51.3	4	100.0	0	0.0	0.0	49.0	18	100.0	1	5.6	1.0	25.8

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR3AE_SE_01JUL2021_40703.xls
31JAN2022 8:41

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 4
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR4AE_SE_01JUL2021_40703.xls
31JAN2022 8:42

POPULATION: Safety-Evaluable Population
ENDPOINT: AEs Grade 5 (AEs leading to Death)
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

Null Report: No results could be derived for this output.

Only AEs of highest severity are counted.
To the SOC Overall row counts, a patient contributes only with the AE occuring with the highest grade within the SOC.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw_soc.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_soc_WGR5AE_SE_01JUL2021_40703.xls
31JAN2022 8:44

POPULATION: Safety-Evaluable Population
ENDPOINT: Case of an elevated ALT or AST in combination with either an elevated Bilirubin or clinical jaundice, as defined in protocol
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_ALTAST_SE_01JUL2021_40703.xls
28JAN2022 15:00

POPULATION: Safety-Evaluable Population
ENDPOINT: Suspected transmission of an infectious agent by the study drug
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_INFAG_SE_01JUL2021_40703.xls
28JAN2022 15:04

POPULATION: Safety-Evaluable Population
ENDPOINT: Serious case of an elevated ALT or AST in combination with either an elevated Bilirubin or clinical jaundice, as defined in protocol
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_SALTAST_SE_01JUL2021_40703.xls
28JAN2022 15:03

POPULATION: Safety-Evaluable Population
ENDPOINT: Serious suspected transmission of an infectious agent by the study drug
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_SINFAG_SE_01JUL2021_40703.xls
28JAN2022 15:07

POPULATION: Safety-Evaluable Population
ENDPOINT: Case of an elevated ALT or AST in combination with either an elevated Bilirubin or clinical jaundice, as defined in protocol Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AESIs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WG35ALTAST_SE_01JUL2021_40703.xls
28JAN2022 15:02

POPULATION: Safety-Evaluable Population
ENDPOINT: Suspected transmission of an infectious agent by the study drug Grade 3-5
MODEL: Unstratified analysis
STUDY: BN40703
Dichotomous Analysis (Safety)

		Patients with 2 SMN2 copies (N=7)						Patients with 3 SMN2 copies (N=7)						Patients with >=4 SMN2 copies (N=4)						All Patients (N=18)					
		Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event				Patients		Patients with Event			
Name	Level	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)	n	%	n	%	95% CI (LL) (Wilson)	95% CI (UL) (Wilson)
All	n/a	7	100.0	0	0.0	0.0	35.4	7	100.0	0	0.0	0.0	35.4	4	100.0	0	0.0	0.0	49.0	18	100.0	0	0.0	0.0	17.6

Only AESIs of highest severity are counted.
95% CI based on Wilson Scores.
Clinical cut-off: 01JUL2021

Program: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_Base/prod/program/t_ae_raw.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_raw_WG35INFAG_SE_01JUL2021_40703.xls
28JAN2022 15:06

POPULATION: Safety-Evaluable Population
STUDY: BN40703
Outcome of Adverse Events

	Patients with 2 SMN2 copies (N=7)														
	Total	RECOVERED/RESOLVED		RECOVERED/RESOLVED WITH SEQUELAE		NOT RECOVERED/NOT RESOLVED		FATAL		RECOVERING/RESOLVING		UNKNOWN		MISSING	
Endpoint Grade	n	n	%	n	%	n	%	n	%	n	%	n	%	n	%
Grade 3-5 AEs															
All	1	1	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
Grade 3	1	1	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0

Clinical cut-off: 01JUL2021

Program: root/global/globalshare/morse_macros/current/prod/program/t_ae_resolved.sas
Output: root/clinical_studies/RO7034067/CDPT7916/BN40703/data_analysis/ACE_eSub_sNDA_analysis/prod/output/t_ae_resolved_SE_01JUL2021_40703.xls
14JUL2022 12:19

Patients with 3 SMN2 copies (N=7)														
Total	RECOVERED/RESOLVED		RECOVERED/RESOLVED WITH SEQUELAE		NOT RECOVERED/NOT RESOLVED		FATAL		RECOVERING/RESOLVING		UNKNOWN		MISSING	
n	n	%	n	%	n	%	n	%	n	%	n	%	n	%
1	1	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
1	1	100.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0

Patients with >=4 SMN2 copies (N=4)														
Total	RECOVERED/RESOLVED		RECOVERED/RESOLVED WITH SEQUELAE		NOT RECOVERED/NOT RESOLVED		FATAL		RECOVERING/RESOLVING		UNKNOWN		MISSING	
n	n	%	n	%	n	%	n	%	n	%	n	%	n	%
0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0
0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0	0	0.0