

## Supplemental Materials

**Table S1. Patient demographics/characteristics by treatment distribution**

Demographics/ characteristics	Patients, n (%)								
	Monotherapy			Add-on and Combination Treatment Scenarios					All Patients in the Add-on and Combination Treatment Scenarios (n=187)
	Onasemnogene Abepravovec (n=194)	Nusinersen (n=49)	Risdiplam (n=9)	Add-on (n=32)	Transient Add-on (n=5)	Combination with Onasemnogene Abepravovec (n=25)	Bridge to Onasemnogene Abepravovec (n=58)	Switch to Onasemnogene Abepravovec (n=67)	
<b>Sex, n (%)</b>									
Male	92 (47.4)	24 (49.0)	5 (55.6)	11 (34.4)	3 (60.0)	10 (40.0)	24 (41.4)	32 (47.8)	80 (42.7)
Female	102 (52.6)	25 (51.0)	4 (44.4)	21 (65.6)	2 (40.0)	15 (60.0)	34 (58.6)	35 (52.2)	107 (57.2)
<b>Country, n (%)</b>									
United States	153 (78.9)	34 (69.4)	8 (88.9)	29 (90.7)	5 (100.0)	23 (92.0)	33 (56.9)	34 (50.8)	124 (66.3)
Greece	4 (2.1)	4 (8.2)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	3 (4.5)	3 (1.6)
Ireland	0 (0)	2 (4.1)	0 (0)	0 (0)	0 (0)	0 (0)	1 (1.7)	4 (6.0)	5 (2.7)
Israel	3 (1.6)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	1 (1.7)	2 (3.0)	3 (1.6)
Japan	20 (10.3)	3 (6.1)	0 (0)	1 (3.1)	0 (0)	2 (8.0)	20 (34.5)	13 (19.4)	36 (19.3)
Portugal	5 (2.6)	2 (4.1)	0 (0)	0 (0)	0 (0)	0 (0)	3 (5.2)	3 (4.5)	6 (3.2)
Romania	0 (0)	1 (2.0)	0 (0)	1 (3.1)	0 (0)	0 (0)	0 (0)	0 (0)	1 (0.5)
Russia	5 (2.6)	1 (2.0)	1 (11.1)	0 (0)	0 (0)	0 (0)	0 (0)	4 (6.0)	4 (2.1)

Patients, n (%)									
Demographics/ characteristics	Monotherapy			Add-on and Combination Treatment Scenarios					All Patients in the Add-on and Combination Treatment Scenarios (n=187)
	Onasemnogene Abeprarvovec (n=194)	Nusinersen (n=49)	Risdiplam (n=9)	Add-on (n=32)	Transient Add-on (n=5)	Combination with Onasemnogene Abeprarvovec (n=25)	Bridge to Onasemnogene Abeprarvovec (n=58)	Switch to Onasemnogene Abeprarvovec (n=67)	
South Korea	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	2 (3.0)	2 (1.1)
Taiwan	4 (2.1)	2 (4.1)	0 (0)	1 (3.1)	0 (0)	0 (0)	0 (0)	2 (3.0)	3 (1.6)
<b>Number of SMN2 copies, n (%)</b>									
One	4 (2.1)	0 (0)	0 (0)	0 (0)	0 (0)	1 (4.0)	0 (0)	0 (0)	1 (0.5)
Two	91 (46.9)	8 (16.3)	4 (44.5)	26 (81.3)	5 (100.0)	23 (92.0)	39 (67.2)	51 (76.1)	144 (77.1)
Three	79 (40.7)	30 (61.2)	3 (33.3)	6 (18.8)	0 (0)	1 (4.0)	17 (29.3)	16 (23.9)	40 (21.4)
Four	12 (6.2)	10 (20.4)	2 (22.2)	0 (0)	0 (0)	0 (0)	1 (1.7)	0 (0)	1 (0.5)
More than four	7 (3.6)	1 (2.1)	0 (0)	0 (0)	0 (0)	0 (0)	1 (1.7)	0 (0)	1 (0.5)
Missing	1 (0.5)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)
<b>SMA type, n (%)</b>									
0	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	1 (4.0)	0 (0)	0 (0)	1 (0.5)
1	85 (43.8)	8 (16.3)	3 (33.3)	27 (84.4)	5 (100.0)	23 (92.0)	35 (60.3)	54 (80.6)	144 (77.0)
2	25 (12.9)	13 (26.5)	2 (22.2)	3 (9.4)	0 (0)	1 (4.0)	9 (15.5)	7 (10.5)	20 (10.7)
3	4 (2.1)	23 (47.0)	1 (11.1)	2 (6.3)	0 (0)	0 (0)	0 (0)	0 (0)	2 (1.1)

Patients, n (%)									
Demographics/ characteristics	Monotherapy			Add-on and Combination Treatment Scenarios					All Patients in the Add-on and Combination Treatment Scenarios (n=187)
	Onasemnogene Abeprarvovec (n=194)	Nusinersen (n=49)	Risdiplam (n=9)	Add-on (n=32)	Transient Add-on (n=5)	Combination with Onasemnogene Abeprarvovec (n=25)	Bridge to Onasemnogene Abeprarvovec (n=58)	Switch to Onasemnogene Abeprarvovec (n=67)	
Missing	80 (41.2)	5 (10.2)	3 (33.3)	0 (0)	0 (0)	0 (0)	14 (24.1)	6 (9.0)	20 (10.7)
<b>Identified by newborn screening, n (%)</b>	113 (58.3)	7 (14.2)	0 (0)	15 (46.9)	2 (40.0)	7 (28.0)	17 (29.3)	15 (22.4)	56 (29.9)
<b>Symptomatic at diagnosis, n (%)</b>	101 (52.1)	44 (89.8)	7 (77.8)	26 (81.3)	4 (80.0)	25 (100.0)	44 (75.9)	58 (87.6)	157 (83.9)
<b>Age at initial SMA diagnosis, months</b>									
Median (range)	1 (0, 27)	32 (0, 502)	14 (3, 105)	2 (0, 22)	5 (0, 5)	2 (0, 17)	2.5 (0, 22)	5 (0, 23)	3 (0, 23)
IQR	0–6	9–78	8–34	0–5	4–5	1–4	0–8	1–8	1–7
Mean (SD)	4.1 (5.98)	68.3 (96.9)	27.1 (31.7)	4.13 (6.1)	3.8 (2.2)	3.52 (4.2)	4.98 (5.8)	5.66 (5.4)	4.8 (5.4)
<b>Age at symptom onset, months</b>									
Median (range)	2 (0, 23)	15 (0, 168)	5 (0, 36)	2 (0, 14)	2 (0, 5)	1 (0, 8)	2 (0, 10)	2 (0, 9)	2 (0, 14)
IQR	1–6	6–36	3–12	0–3	1–3	1–3	0–4	1–5	0–4

Patients, n (%)									
Demographics/ characteristics	Monotherapy			Add-on and Combination Treatment Scenarios					All Patients in the Add-on and Combination Treatment Scenarios (n=187)
	Onasemnogene Abeparvovec (n=194)	Nusinersen (n=49)	Risdiplam (n=9)	Add-on (n=32)	Transient Add-on (n=5)	Combination with Onasemnogene Abeparvovec (n=25)	Bridge to Onasemnogene Abeparvovec (n=58)	Switch to Onasemnogene Abeparvovec (n=67)	
Mean (SD)	4.3 (4.9)	29.7 (39.3)	10.4 (12.1)	2.0 (2.7)	2.2 (1.9)	2.2 (2.5)	2.7 (3.2)	2.6 (2.2)	2.4 (2.6)
Missing, n	69	5	2	0	0	0	14	5	19
<b>Age at first treatment, months</b>									
Median (range)	3 (0, 54)	37 (0, 507)	23 (3, 106)	3 (0, 23)	6 (1, 7)	3 (0, 17)	3 (0, 22)	6 (0, 21)	3 (0, 23)
IQR	1–9	13–81	8–34	1.5–7	6–6	2–5	2–8	2–13	2–9
Mean (SD)	6.5 (8.4)	72.8 (97.9)	29.2 (31.4)	6.1 (6.6)	5.2 (2.3)	4.2 (3.8)	5.5 (5.8)	7.6 (6.1)	6.2 (5.9)
<b>Interval between diagnosis and treatment, months</b>									
Median (range)	1 (0, 48)	2 (0, 37)	1 (0, 9)	1 (0, 16)	1 (1, 2)	1 (0, 3)	0 (0, 3)	1 (0, 20)	1 (0, 20)
IQR	1–2	1–4	0–2	1–1.5	1–2	0–1	0–1	0–2	0–1
Mean (SD)	2.4 (6.2)	4.5 (7.2)	2.1 (3.2)	2.0 (3.5)	1.4 (0.5)	0.7 (0.7)	0.6 (0.8)	2.2 (3.8)	1.4 (2.8)
<b>Weight at onasemnogene abeparvovec administration, kg</b>									
Median (Min, Max)	5.2 (1.6, 12.5)	N/A	N/A	5.7 (3.2, 12.2)	5.8 (4.1, 7)	8.0 (2.6, 12.0)	7.1 (3.1, 11.9)	8.5 (4.2, 15.0)	7.5 (2.6, 15)

Patients, n (%)									
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IQR	4–7.8	N/A	N/A	4.5–7.8	5.1–6.3	6.3–9.7	5.9–8.75	7.3–9.65	(6.1–9.2)
Mean (SD)	5.9 (2.3)	N/A	N/A	6.4 (2.4)	5.7 (1.1)	8.0 (2.2)	7.4 (1.9)	8.4 (1.9)	7.6 (2.2)
Missing, n	13	N/A	N/A	3	0	0	6	7	16
<b>Age at onasemnogene abeparvovec administration, months</b>									
Median (Min, Max)	3 (0, 54)	N/A	N/A	3 (0, 23)	6 (1, 7)	9 (1, 31)	7 (1, 45)	19 (2, 38)	9 (0, 45)
IQR	1–9	N/A	N/A	1.5–7	6–6	5–18	5–13	12–23	5–20
Mean (SD)	6.5 (8.4)	N/A	N/A	6.1 (6.6)	5.2 (2.4)	12.0 (8.4)	9.7 (7.7)	17.7 (7.5)	12.1 (8.7)
<b>Duration of follow up after onasemnogene abeparvovec administration, months</b>									
Median (range)	14.98 (0.03, 49.2)	N/A	N/A	25.9 (13.3, 40.2)	29.0 (21.1, 34.7)	18.3 (0.9, 46.6)	20.8 (0.4, 44.6)	23.6 (0.3, 39.6)	23.0 (0.3, 46.6)
IQR	7–23.8	N/A	N/A	19.5–32.1	25.7–31.1	11.7–35.4	13.1–25.8	15.3–28.4	15.3–29.3
Mean (SD)	15.8 (10.3)	N/A	N/A	26.5 (7.8)	28.3 (5.2)	22.8 (14.2)	19.9 (10.1)	21.5 (9.7)	22.2 (10.4)

IQR, interquartile range; N/A, not applicable; SD, standard deviation; SMA, spinal muscular atrophy; *SMN2*, survival motor neuron 2 gene.