Interim Results From the Ongoing RESPOND Study Evaluating Nusinersen in Children With Spinal Muscular Atrophy Previously Treated With Onasemnogene Abeparvovec

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- **CP:** advisory boards and consultant for AveXis, Biogen, and Sarepta; speaker for AveXis and Biogen; Principal Investigator of clinical studies for Astellas, AveXis, Biogen, Catabasis, CSL Behring, Pfizer, PTC, Sarepta, and Scholar Rock
- **JAP:** advisor and consultant for AveXis, Biogen, and Sarepta; Principal Investigator of clinical studies for AveXis, Biogen, Pfizer, PTC, Sarepta, and Scholar Rock
- **NLK:** advisory boards for Argenx, Astellas, AveXis, Biogen, Cytokinetics, PTC, Roche, and Sarepta; honoraria from Biogen; clinical research funding direct to institution from Astellas, AveXis, Biogen, and Sarepta
- **JFB:** consultant for Audentes, AveXis, Biogen, Cytokinetics, Genentech, Marathon, Momenta, NS Pharma, PTC Therapeutics, Sarepta, Scholar Rock, and WaVe; speaker for AveXis and Biogen; medical advisory council member for Cure SMA; site investigator for clinical trials with Alexion, AveXis, Biogen, Catabasis, CSL Behring, Cytokinetics, Fibrogen, Pfizer, PTC Therapeutics, Sarepta, Summit, and WaVe
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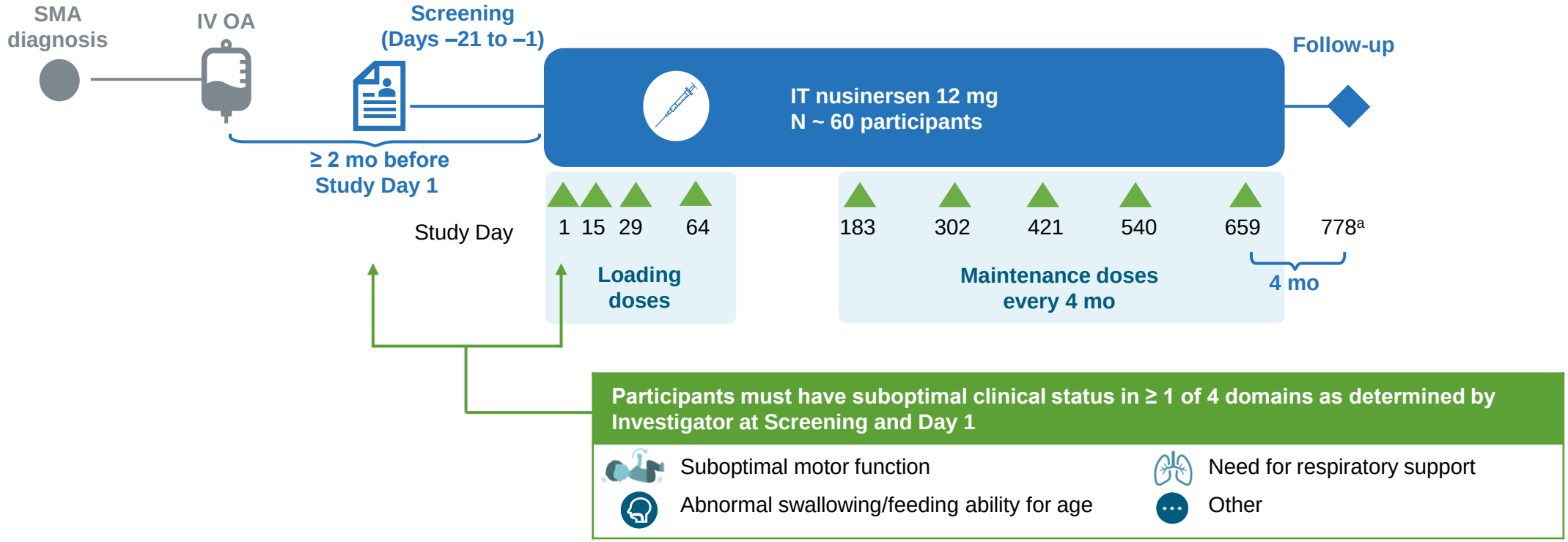
RESPOND study design

- RESPOND is a Phase 4, multicenter, single-arm, open-label study evaluating nusinersen in participants with SMA who have previously received OA and have suboptimal clinical status as determined by the Investigator

- **Analysis objective:** Provide baseline characteristics and interim clinical outcomes and safety findings for participants enrolled in RESPOND



IT = intrathecal; IV = intravenous; OA = onasemnogene abeparvovec; SMA = spinal muscular atrophy
The study (NCT04488133) design as shown reflects a protocol amendment. For the 15 November 2022 data cut, participants were enrolled either under the original protocol (requiring participants to have received onasemnogene abeparvovec ≥ 3 months before Study Day 1) or the protocol amendment (requiring participants to have received onasemnogene abeparvovec ≥ 2 months before Study Day 1). [a]Or 4 months from last dose.

RESPOND key eligibility criteria

Key eligibility criteria

Inclusion criteria[a]

- Age ≤ 36 mo at first nusinersen dose
- ≥ 1 *SMN2* copies
- Received IV OA ≥ 2 mo before first dose of nusinersen
- Suboptimal clinical status per the Investigator[b]

Exclusion criteria

- Severe or serious AEs related to OA ongoing at Screening
- Permanent ventilation[c]
- Previously received nusinersen

Additional inclusion criteria for Subgroups A and B

Subgroup A

- Aged ≤ 9 mo at first nusinersen dose
- 2 *SMN2* copies
- SMA symptom onset at age ≤ 4 mo
- Received IV OA at age > 6 wk to ≤ 6 mo and after SMA symptom onset

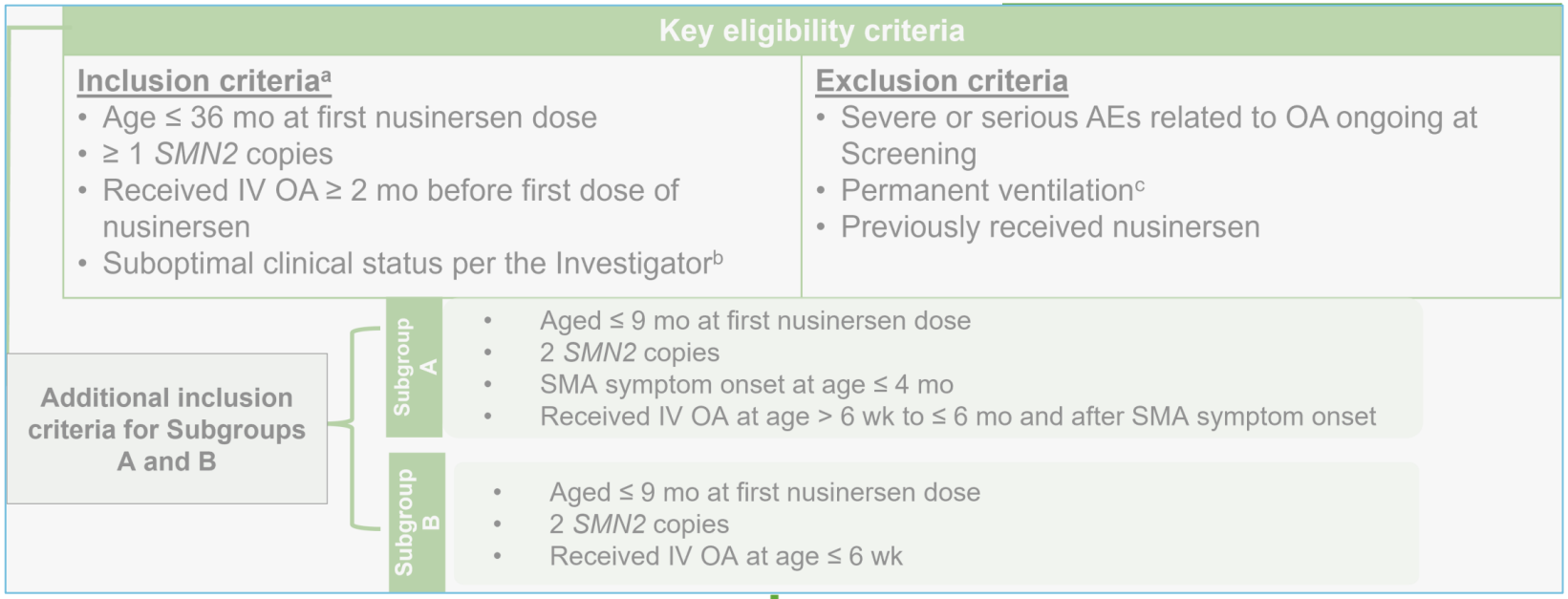
Subgroup B

- Aged ≤ 9 mo at first nusinersen dose
- 2 *SMN2* copies
- Received IV OA at age ≤ 6 wk

AE = adverse event; IV = intravenous; OA = onasemnogene abeparvovec; SMA = spinal muscular atrophy; *SMN2* = survival motor neuron 2

[a]Additional criterion: alanine transaminase or aspartate transaminase ≤ 2 × upper limit of normal at Screening and within 7 days before dosing. The key eligibility criteria shown reflect protocol amendments, which also included the addition of Subgroup B. For the 15 November 2022 data cut, participants were enrolled either under the original protocol (requiring participants to have received OA ≥ 3 months before Study Day 1) or the protocol amendment (requiring participants to have received onasemnogene abeparvovec ≥ 2 months before Study Day 1). [b]Suboptimal clinical status in ≥ 1 of the following domains: suboptimal motor function, need for respiratory support, abnormal swallowing function or feeding ability for age, or any other suboptimal clinical status. [c]Tracheostomy or ≥ 16 hours ventilation/day continuously for > 21 days in the absence of an acute reversible event. [d]Participants in this analysis group received their first dose of nusinersen at < 300 days of age. [e]All enrolled participants have 2 or 3 *SNM2* copies as of the 15 November 2022 data cut.

RESPOND key eligibility criteria and analysis groups



Key RESPOND analysis groups

Age ≤ 9 mo at First Nusinersen Dose

- Age ≤ 9 mo at first nusinersen dose[d]
- 2 *SMN2* copies

Age > 9 mo at First Nusinersen Dose

- Age > 9 mo up to ≤ 36 mo at first nusinersen dose
- ≥ 1 *SMN2* copies[e]

AE = adverse event; IV = intravenous; OA = onasemnogene abeparvovec; SMA = spinal muscular atrophy; *SMN2* = survival motor neuron 2

[a]Additional criterion: alanine transaminase or aspartate transaminase ≤ 2 × upper limit of normal at Screening and within 7 days before dosing. The key eligibility criteria shown reflect protocol amendments, which also included the addition of Subgroup B. For the 15 November 2022 data cut, participants were enrolled either under the original protocol (requiring participants to have received OA ≥ 3 months before Study Day 1) or the protocol amendment (requiring participants to have received onasemnogene abeparvovec ≥ 2 months before Study Day 1). [b]Suboptimal clinical status in ≥ 1 of the following domains: suboptimal motor function, need for respiratory support, abnormal swallowing function or feeding ability for age, or any other suboptimal clinical status. [c]Tracheostomy or ≥ 16 hours ventilation/day continuously for > 21 days in the absence of an acute reversible event. [d]Participants in this analysis group received their first dose of nusinersen at < 300 days of age. [e]All enrolled participants have 2 or 3 *SNM2* copies as of the 15 November 2022 data cut.

Interim analyses

Cutoff date for the interim analysis: 15 November 2022

Interim Set (n = 29)

- Children who received ≥ 1 dose of nusinersen who had the opportunity to complete the Day 183 assessment as of the data cut[a]

Outcomes

- Changes in suboptimal clinical status
- Total HINE-2 score (primary outcome)
- CHOP INTEND

- Analyses stratified by age at first nusinersen dose (≤ 9 mo, > 9 mo) and *SMN*2 copy number

Safety Set (n = 38)

- All children who received ≥ 1 dose of nusinersen as of the data cut

Outcomes

- Safety and tolerability

CHOP INTEND = Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; HINE-2 = Hammersmith Infant Neurological Examination – Section 2; *SMN2* = survival motor neuron 2

[a]One participant with risdiplam use was excluded since there were no available post-baseline data on clinical endpoints when the participant was only treated with nusinersen.

Baseline demographics and SMA history (Interim Set)

Demographics and SMA History	Age at First Nusinersen Dose ≤ 9 mo	Age at First Nusinersen Dose > 9 mo	
	2 *SMN2* Copies n = 14	2 *SMN2* Copies n = 12	3 *SMN2* Copies n = 3
Male / female, n (%)	9 (64.3) / 5 (35.7)	7 (58.3) / 5 (41.7)	3 (100) / 0
Age at SMA symptom onset, median (range), mo	0.8 (0.0-3.0)	1.0 (0.0-5.0)[a,b]	6.0 (5.0-9.0)
Age at SMA diagnosis, median (range), mo	0.9 (0.0-6.0)[c,d]	2.1 (0.9-6.0)[b]	15.0 (6-23.0)
Age at OA dosing, median (range), mo	**1.7 (0.7-5.1)**	**2.7 (0.8-6.9)**	**17.5 (13.6-24.3)**
Participants who were symptomatic at the time of OA dosing, n (%)	14 (100)	12 (100)	3 (100)
Age at first nusinersen dose, median (range), mo	**7.7 (3.4-9.8)[e]**	**16.3 (11.0-33.3)**	**30.8 (29.2-35.7)**
Time from OA dose to first nusinersen dose, median (range), mo	4.8 (2.6-7.7)	14.4 (6.3-31.3)	13.3 (4.9-22.2)

OA = onasemnogene abeparvovec; SMA = spinal muscular atrophy; *SMN2* = survival motor neuron 2
[a]One participant exhibited SMA symptoms on day of birth. The minimum age at SMA symptom onset was 0 days. [b]n = 11; age information was missing for 1 participant. [c]The minimum age at SMA diagnosis was 0 days. [d]n = 13; 1 participant with anomaly for diagnosis age was excluded. [e]Participants in this analysis group received first dose of nusinersen at < 300 days of age.

Baseline disease characteristics (Interim Set)

- The majority of participants had low CMAP ulnar amplitude (≤ 1 mV) at Baseline

Disease Characteristics	Age at First Nusinersen Dose ≤ 9 mo	Age at First Nusinersen Dose > 9 mo	
	2 *SMN2* Copies n = 14	2 *SMN2* Copies n = 12	3 *SMN2* Copies n = 3
Achieved motor milestone and maintained at Screening, n (%)			
Sitting without support	0	7 (58.3)	1 (33.3)
Standing without support	0	0	0
Walking with support	0	0	0
Participants who had prior ventilatory support, n (%)[a]	9 (64.3)	6 (50.0)	0
HINE-2 total score, median (range)	3.0 (0–11)	8.0 (1–18)[b]	12 (6–19)
CHOP INTEND total score, median (range)	37.5 (30–52)	46.0 (37–64)[b,c]	33[c,d]
CMAP ulnar amplitude (mV),[e] median (range)	0.75 (0.19–2.10)	0.66 (0.20–2.70)	0.9 (0.7–5.6)
≤ 1 mV, n (%)	11 (79)	9 (75)	2 (67)
> 1 to ≤ 2 mV, n (%)	2 (14)	2 (17)	0
> 2 to ≤ 5 mV, n (%)	1 (7)	1 (8)	0
> 5 mV, n (%)	0	0	1 (33)
CMAP peroneal amplitude (mV),[f] median (range)	0.75 (0.10–2.00)[b]	1.15 (0.30–2.90)	1.70 (1.5–4.0)
≤ 1 mV, n (%)	9 (64)	5 (42)	0
> 1 to ≤ 2 mV, n (%)	2 (14)	5 (42)	2 (67)
> 2 to ≤ 5 mV, n (%)	0	2 (17)	1 (33)
> 5 mV, n (%)	0	0	0

CHOP INTEND = Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; CMAP = compound muscle action potential; *SMN2* = survival motor neuron 2; HINE-2 = Hammersmith Infant Neurological Examination – Section 2 [a]One participant required ventilatory support ≥ 16 hours/day, 2 were unknown, and the remainder required ventilatory support < 16 hours/day. Bilevel positive airway pressure/continuous positive airway pressure was the most used ventilatory device. [b]n = 11. [c]CHOP INTEND was not administered to 3 participants (1 in the > 9-month, 2 *SMN2* copy group and 2 in the > 9-month, 3 *SMN2* copy group) per protocol because they were ≥ 2 years of age at time of informed consent and achieved sitting. [d]n = 1. [e]Ulnar nerve innervation of abductor digiti minimi. [f]Peroneal nerve innervation of anterior tibialis.

Most participants had Investigator-reported suboptimal clinical status in multiple domains at Baseline

Participants With Suboptimal Clinical Status in Each Domain at Baseline	Age at First Nusinersen Dose ≤ 9 mo	Age at First Nusinersen Dose > 9 mo	
	2 *SMN2* Copies (n = 14)	2 *SMN2* Copies (n = 12)	3 *SMN2* Copies (n = 3)
Motor function	n = 13	n = 11	n = 3
Swallowing or feeding ability for age	n = 9	n = 5	n = 1
Respiratory function	n = 10	n = 9	n = 0

SMN2 = survival motor neuron 2
Two participants aged ≤ 9 months at first nusinersen dose and 1 participant aged > 9 months with 2 *SMN2* copies were reported to have suboptimal clinical status in “other” category at Baseline.

Most participants with Investigator-reported suboptimal motor function at Baseline improved at Day 183

- The majority with suboptimal swallowing/feeding ability or respiratory function at Baseline had no changes; some improved

Motor function

Changes at Day 183, n

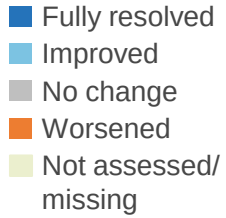
Swallowing or feeding ability for age

Changes at Day 183, n

Respiratory function

Changes at Day 183, n

Participants With Suboptimal Clinical Status in Each Domain at Baseline	Age at First Nusinersen Dose ≤ 9 mo	Age at First Nusinersen Dose > 9 mo	
	2 *SMN2* Copies (n = 14)	2 *SMN2* Copies (n = 12)	3 *SMN2* Copies (n = 3)
Motor function	**n = 13**	**n = 11**	**n = 3**
Changes at Day 183, n	1, 12	1, 10	3
Swallowing or feeding ability for age	**n = 9**	**n = 5**	**n = 1**
Changes at Day 183, n	3, 6	2, 3	1
Respiratory function	**n = 10**	**n = 9**	**n = 0**
Changes at Day 183, n	2, 8	1, 3, 5	



SMN2 = survival motor neuron 2

Two participants aged ≤ 9 months at first nusinersen dose and 1 participant aged > 9 months with 2 *SMN2* copies were reported to have suboptimal status in "other" category at Baseline. At Day 183, no change was reported in 1 of the ≤ 9-month-old participants. Two other participants were not assessed at Day 183.

Most participants had Caregiver-reported suboptimal clinical status in multiple domains at Baseline

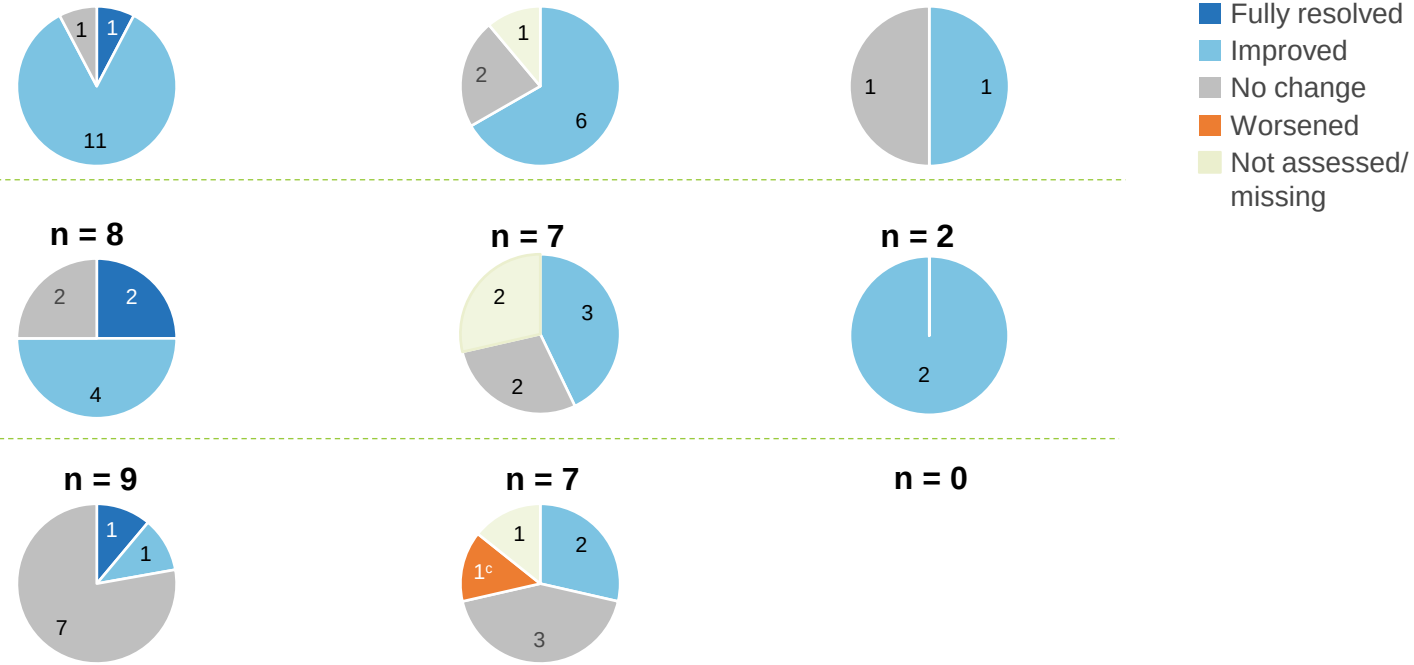
Participants With Suboptimal Clinical Status in Each Domain at Baseline	Age at First Nusinersen Dose ≤ 9 mo; 2 *SMN2* Copies (n = 14)	Age at First Nusinersen Dose > 9 mo	
		2 *SMN2* Copies (n = 12)	3 *SMN2* Copies (n = 3)
Motor function[a]	n = 13	n = 9	n = 2
Swallowing or feeding ability for age	n = 8	n = 7	n = 2
Respiratory function[b]	n = 9	n = 7	n = 0

SMN2 = survival motor neuron 2
Two participants aged ≤ 9 months at first nusinersen dose and 1 participant aged > 9 months at first nusinersen dose with 2 *SMN2* copies was reported to have suboptimal status in "other" category at Baseline.
[a]Language used on form for parents/caregivers was "Strength and ability to move." [b]Language used on form for parents/caregivers was "Ability to breath."

Most participants with Caregiver-reported suboptimal motor function at Baseline improved at Day 183

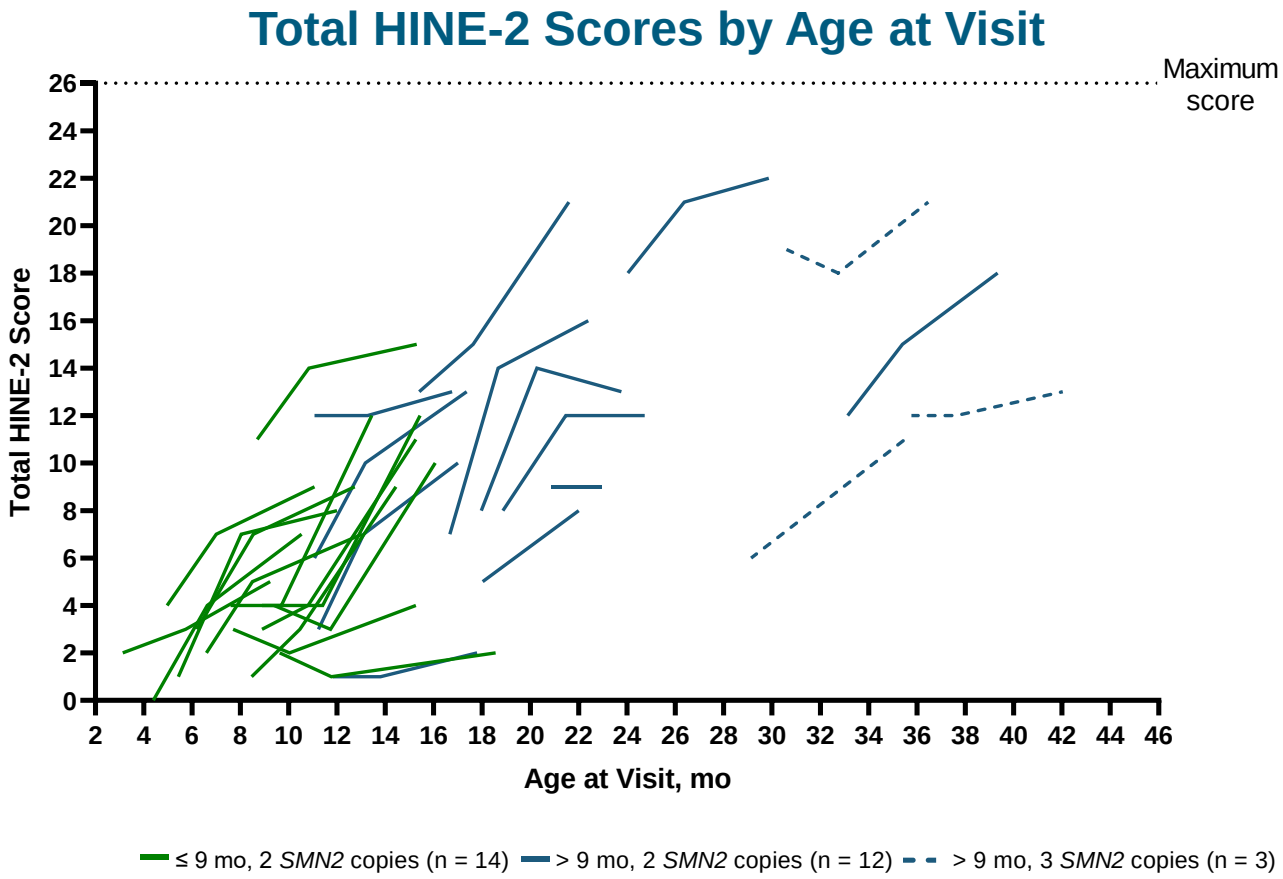
- Many with suboptimal swallowing/feeding ability at Baseline improved
- The majority with suboptimal respiratory function at Baseline had no changes

Participants With Suboptimal Clinical Status in Each Domain at Baseline	Age at First Nusinersen Dose ≤ 9 mo; 2 *SMN2* Copies (n = 14)	Age at First Nusinersen Dose > 9 mo	
		2 *SMN2* Copies (n = 12)	3 *SMN2* Copies (n = 3)
Motor function[a] Changes at Day 183, n	**n = 13**	**n = 9**	**n = 2**
Swallowing or feeding ability for age Changes at Day 183, n	**n = 8**	**n = 7**	**n = 2**
Respiratory function[b] Changes at Day 183, n	**n = 9**	**n = 7**	**n = 0**



Two participants aged ≤ 9 months at first nusinersen dose and 1 participant aged > 9 months at first nusinersen dose with 2 *SMN2* copies were reported to have suboptimal status in "other" category at Baseline. At Day 183, 1 participant aged ≤ 9 months at first nusinersen dose was reported to have "improved," and the other 2 participants were "not assessed." [a]Language used on form for caregivers was "Strength and ability to move." [b]Language used on form for caregivers was "Ability to breathe." [c]Participant had 2 severe adverse events (acute/chronic respiratory failure and a chronic respiratory failure) in < 2 months prior to Day 183. Both events were unrelated to the study drug and were resolved.

Mean total HINE-2 scores increased from Baseline to Day 183



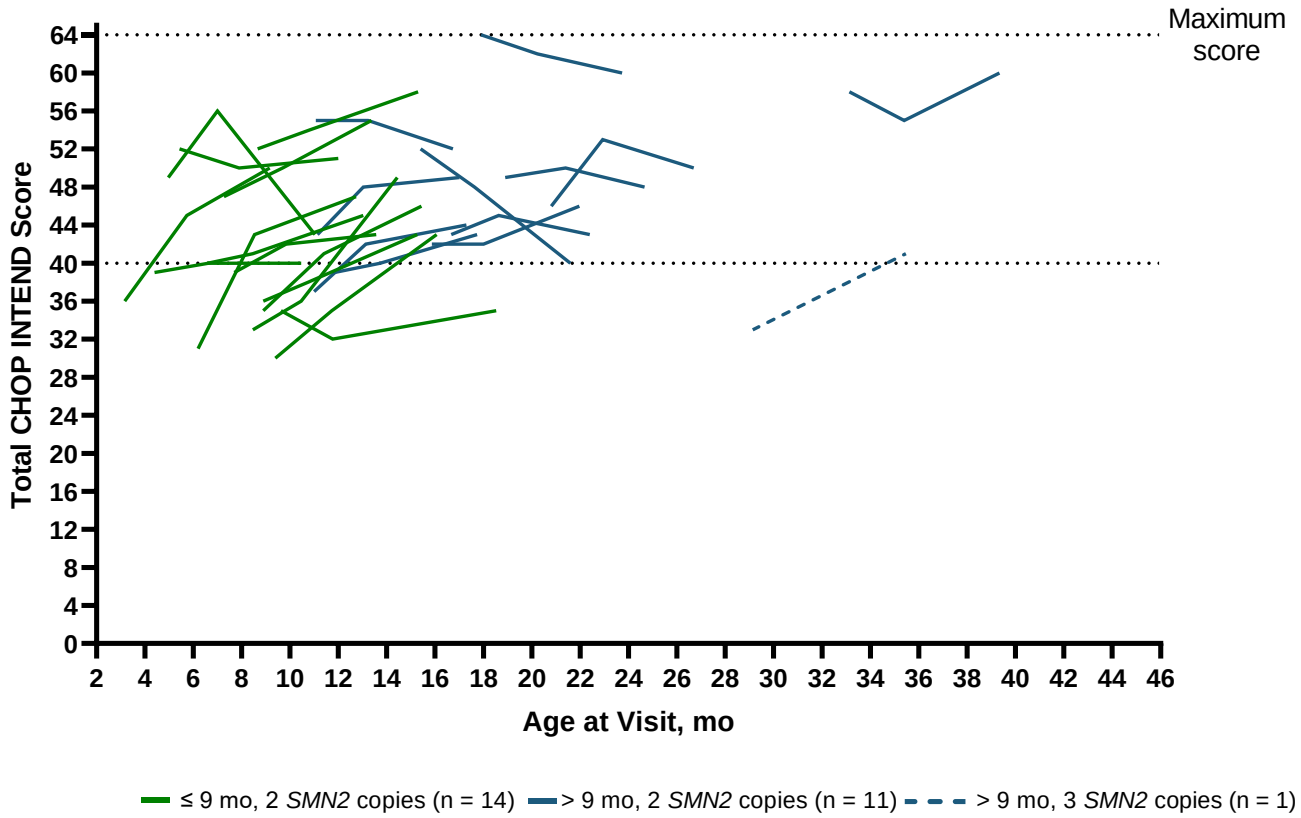
Age at First Nusinersen Dose	Actual Mean (SD) HINE-2 Scores				Mean (SD) Change from Baseline to Day 183
	n	Baseline	n	Day 183	n
≤ 9 mo, 2 *SMN2* Copies	14	3.1 (2.60)	14	8.6 (3.46)	14	5.4 (2.62)
> 9 mo, 2 *SMN2* Copies	11	8.8 (4.79)	11	13.5 (5.77)	10	5.2 (2.74)

Mean changes were not calculated for participants with 3 copies of *SMN2* due to small sample size

HINE-2 = Hammersmith Infant Neurological Examination – Section 2; *SMN2* = survival motor neuron 2
All data from Baseline to Day183 are shown in the figure for participants with post-Baseline scores. One participant each was missing Baseline data (> 9 months, 2 *SMN2* copies), Day 64 (> 9 months, 3 *SMN2* copies), and Day 183 (> 9 months, 2 *SMN2* copies).

Mean total CHOP INTEND scores increased from Baseline to Day 183 in younger group and remained stable in older group

Total CHOP INTEND Scores by Age at Visit



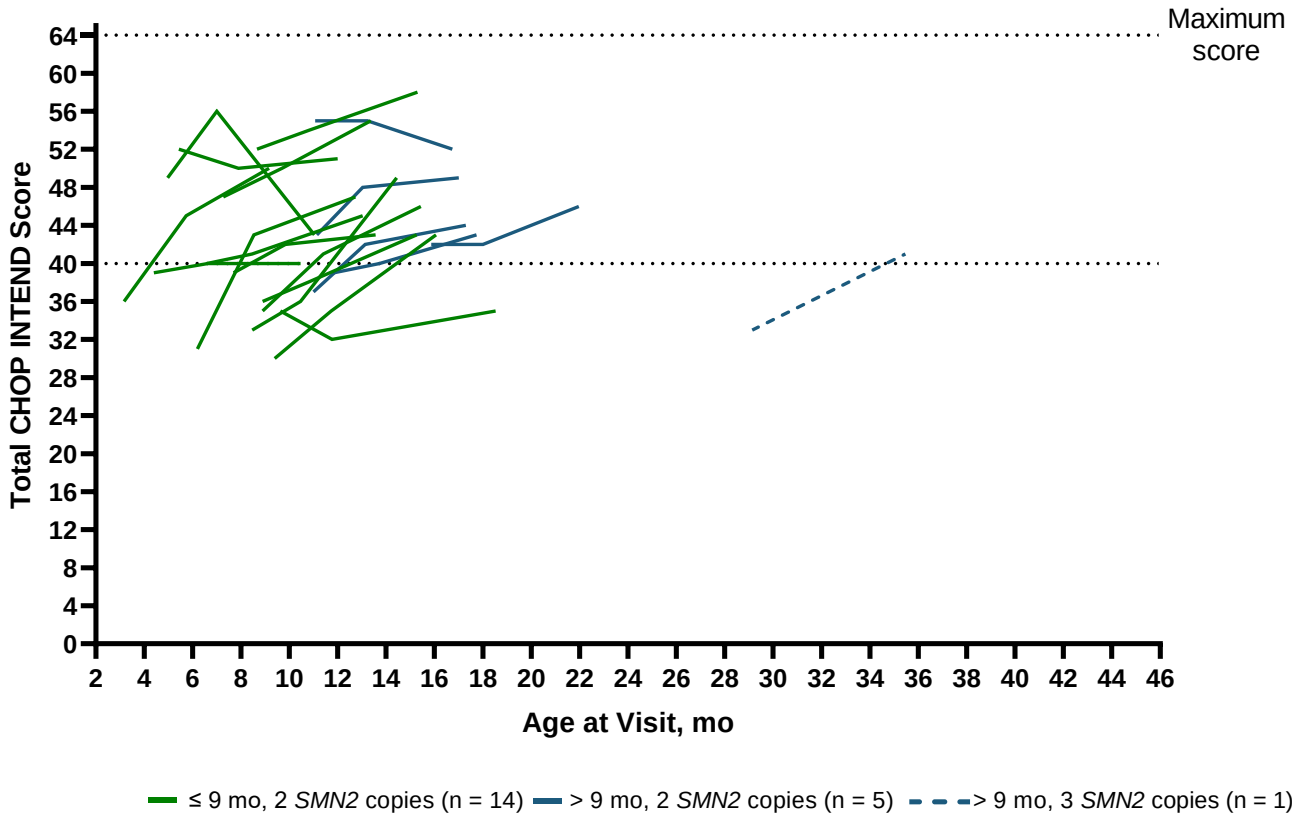
Age at First Nusinersen Dose	Actual Mean (SD) CHOP INTEND Scores				Mean (SD) Change from Baseline to Day 183
	n	Baseline	n	Day 183	n
≤ 9 mo, 2 *SMN2* Copies	14	39.6 (7.49)	14	46.3 (6.01)	14	6.7 (6.76)
> 9 mo, 2 *SMN2* Copies	11	48.0 (8.45)	11	48.6 (6.62)	11	0.6 (5.50)

Mean changes were not calculated for participants with 3 copies of *SMN2* due to small sample size

CHOP INTEND = Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; *SMN2* = survival motor neuron 2
All data from Baseline to Day183 are shown in the figure for participants with post-Baseline scores. CHOP INTEND was not administered to 3 participants per protocol because they were ≥ 2 years of age at time of informed consent and had attained sitting without support. All were age > 9 months at first nusinersen dose, 1 had 2 *SMN2* copies, and 2 had 3 *SMN2* copies . One participant (> 9 months, 3 *SMN2* copies) was missing Day 64 data.

Mean total CHOP INTEND scores increased from Baseline to Day 183 among those unable to sit without support at Screening

Total CHOP INTEND Scores by Age at Visit Among Those Unable to Sit Without Support at Screening



Age at First Nusinersen Dose	Actual Mean (SD) CHOP INTEND Scores				Mean (SD) Change from Baseline to Day 183
	n	Baseline	n	Day 183	n
≤ 9 mo, 2 *SMN2* Copies, Nonsitters at Screening	14	39.6 (7.49)	14	46.3 (6.01)	14	6.7 (6.76)
> 9 mo, 2 *SMN2* Copies, Nonsitters at Screening	5	43.2 (7.01)	5	46.8 (3.70)	5	3.6 (3.91)

Mean changes were not calculated for participants with 3 copies of *SMN2* due to small sample size

CHOP INTEND = Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; *SMN2* = survival motor neuron 2
All data from Baseline to Day183 are shown in the figure for participants unable to sit without support at screening with post-Baseline scores. One participant (> 9 mo, 3 *SMN2* copies) was missing Day 64 data.

Adverse events reported after a median of ~230 days on study (Safety Set)

Safety Parameters, n (%)	Overall Population N = 38
Participants dosed	38 (100)
Time on study, median (range), d	230.5 (28.0–677.0)
Participant with any AE	31 (81.6)
AE related to study drug	
Mild	2 (5.3)[a]
Moderate	0
Severe	0
Serious AE	13 (34.2)[b]
AE leading to study or drug withdrawal	0[c]
Death	0

Most Common AEs by System Organ Class, n (%)	Overall Population N = 38
Infections and infestations	24 (63.2)
Respiratory, thoracic, and mediastinal disorders	10 (26.3)
Gastrointestinal disorders	5 (13.2)
General disorders and administration site conditions	5 (13.2)
Skin and subcutaneous tissue disorders	5 (13.2)

Proportion of Participants With Any AEs and Severity, n (%)	Overall Population N = 38
No AEs	7 (18.4)
Mild AEs	16 (42.1)
Moderate AEs	8 (21.1)
Severe AEs[d]	7 (18.4)

- No serious AEs were considered related to nusinersen
- No clinically significant trends related to nusinersen in hematology, blood chemistry, urinalysis, coagulation, vital signs, ECGs, or liver function tests were observed

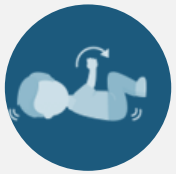
AE = adverse event; ECG = electrocardiogram

[a]Mild AEs of proteinuria in 2 participants were considered related to the study drug by the Investigator. A nephrology consultation was not required. The events resolved and the participants continue to receive nusinersen treatment. [b]None of the serious AEs were considered related to study drug, and all events were resolved. All participants continued treatment with nusinersen. [c]One participant (2.6%) discontinued after first dose of nusinersen due to parent/guardian decision. [d]Severe AEs: acute respiratory failure, chronic respiratory failure, COVID-19, enterovirus infection, feeding intolerance, metapneumovirus pneumonia, Moraxella infection, pneumonia viral, pyrexia, respiratory failure, respiratory syncytial virus infection, and viral upper respiratory infections.

Summary & Conclusions

At Day 183:

- **Most participants with suboptimal motor function at Baseline improved**
- **Many with Caregiver-reported suboptimal swallowing/feeding ability at Baseline improved; Investigators most frequently reported no change in this domain**
- **Many with suboptimal respiratory function had no changes but some improved**



- **Mean total HINE-2 scores increased across age groups**
- **On average, younger participants and older participants who were unable to sit without support improved from Baseline on CHOP INTEND**

No emerging safety concerns have been identified at the time of the data cut in enrolled participants who received nusinersen after OA

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CHOP INTEND = Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; HINE-2 = Hammersmith Infant Neurological Examination – Section 2; OA = onasemnogene abeparvovec