Time to Treatment Effect, Event-free Survival, and Motor Milestone Achievement in Spinal Muscular Atrophy Type 1 Patients Treated With Onasemnogene Abeparvovec (Formerly AVXS-101) Contrasted to Natural History

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Onasemnogene abeparvovec is not approved outside of the US.

1

Disclosures

- AveXis, Inc., a Novartis company, sponsored the AVXS-101-CL-101 (START, NCT02122952) and AVXS-101-CL-303 (STR1VE, NCT03306277) trials
- I am an employee of AveXis, Inc., and own Novartis stock or other equities
- DMS, DEF, FGO, MD, FK, AN, and RA are also employees of AveXis, Inc., and may own Novartis stock or other equities
- MM is a contract employee of AveXis, Inc.
- BM is an employee of SSI Strategy, who are contracted to support AveXis, Inc.

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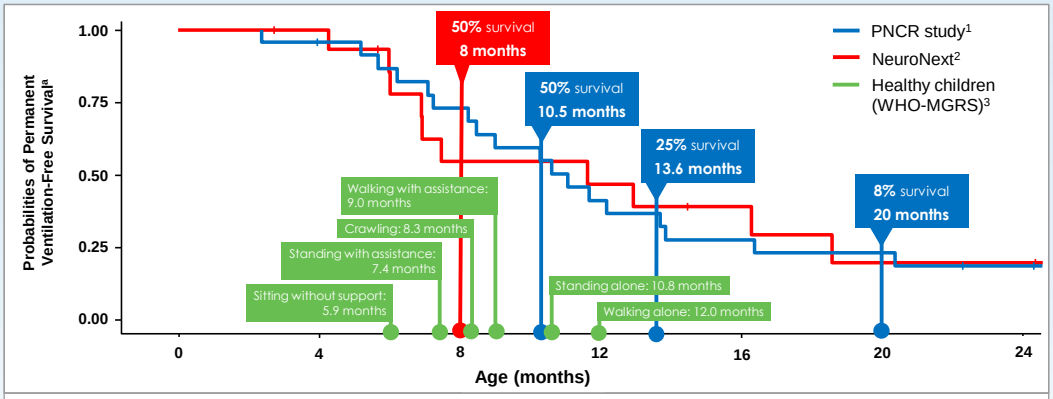
SMA1 Is a Rapidly Progressing Disease Resulting in Death or Permanent Ventilation by 2 Years



[a]**Survival for PNCR[1]** = alive without the need for permanent ventilation, defined as ≥16-hour/day continuously for ≥2 weeks in the absence of an acute reversible illness; n=23 (2 copies of *SMN2*)

Survival for NeuroNext[2] = alive without tracheostomy; n=20

PNCR, Pediatric Neuromuscular Clinical Research; SMA1, spinal muscular atrophy type 1; SMN, survival motor neuron; WHO-MGRS; World Health Organisation Multicentre Growth Reference Study.

1. Finkel RS, et al. *Neurology*. 2014;83:810–817. 2. Kolb SJ, et al. *Ann Neurol*. 2017;82:883–891. 3. WHO-MGRS Group. *Act Pediatrica*. 2006;suppl 450:86–95.

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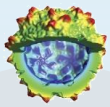
Onasemnogene Abeparvovec (Formerly AVXS-101)

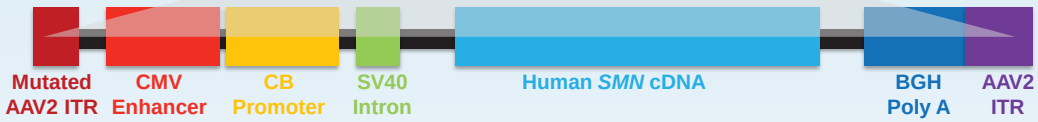
A One-Time *SMN* GRT That Addresses the Genetic Root Cause of SMA

Able to deliver across the blood-brain barrier and into the spinal cord



Recombinant AAV9 Capsid Shell

Designed not to integrate into genome of the patient



Continuous Promoter	Human *SMN* Transgene	scAAV ITR
• Hybrid CMV enhancer and CB promoter activates the transgene to allow for continuous and sustained SMN protein expression	• Full copy of a stable, functioning human *SMN* gene that is introduced into the cell's nucleus	• The scAAV ITR increases the speed at which the double-stranded transgene is transcribed and the resulting protein is produced

AAV2, adeno-associated virus serotype 2; AAV9, AAV serotype 9; BGH Poly A, bovine growth hormone polyadenylation; CB, chicken β-actin; cDNA, complementary DNA; CMV, cytomegalovirus; GRT, gene-replacement therapy; ITR, inverted terminal repeat; scAAV, self-complementary AAV; SMA, spinal muscular atrophy; *SMN*, survival motor neuron gene; SV40, simian virus 40.

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Key Clinical Trial Characteristics of START and STR1VE

	START, CL-101 (NCT02122952)[1,2]	STR1VE, CL-303 (NCT03306277)
Phase	1/2a	3
Median study period time	24 months	10.1 months[a]
Last (end-of-trial) visit	24 months[b] (post-dose)	18 months (of age)
Age eligible for study enrollment	<6 months[c]	<6 months
Total number of patients	Cohort 2: 12 (high dose)	22
Design	A single-arm, open-label study	

START has completed. STR1VE is ongoing.

[a]Data reported as of March 8, 2019; [b]At the time of onasemnogene abeparvovec clinical trial publication, 5 patients had not yet reached the 24-month last visit; [c]At the time of treatment, 1 patient was 7.9 months old.

1. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–1722. 2. Lowes LP, et al. *Pediatric Neurology*. 2019. doi: 10.1016/j.pediatrneurol.2019.05.005.

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Patient Characteristics at Baseline

	START (Cohort 2, n=12)[1]	STR1VE (N=22)[a]	START + STR1VE (n=34)
Female sex, n (%)	7 (58)	12 (55)	19 (56)
Mean age			
Symptom onset, months (range)	1.4 (0–3.0)	1.9 (0–4.0)	1.8 (0–4.0)
Genetic diagnosis, days (range)	60 (0–136)[b]	79 (0–163)[b]	72.1 (0–163)[b]
First dose, months (range)	3.4 (0.9–7.9)	3.7 (0.5–5.9)	3.6 (0.5–7.9)
Mean CHOP INTEND score (range)[c]	28.2 (12–50)	32.0 (18–52)	30.6 (12–52)

START has completed. STR1VE is ongoing.

[a]Data reported as of March 8, 2019; [b]Diagnosis was made prenatally, so an age of 0 was reported at the time of genetic diagnosis; [c]Scores on the CHOP INTEND range from 0 to 64, with higher scores indicating better motor function.

CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders.

1. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–1722.

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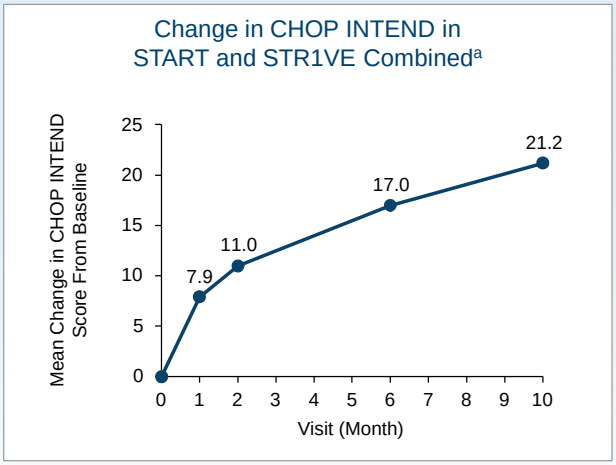
Motor Function Improvements With Onasemnogene Abeparvovec

- A one-time administration of onasemnogene abeparvovec resulted in rapid improvement in motor function, particularly in the first months after dosing
- At 1 month post-dose, CHOP INTEND score increased an average of 7.9 points from baseline

+7.9 points



START has completed. STR1VE is ongoing.

[a]Combined (n=34) includes START (Cohort 2, n=12) and STR1VE (N=22) at Month 1; n=33 (START n=12, STR1VE n=21) at Month 2; n=31 (START n=12, STR1VE n=19) at Month 6; and n=19 (START n=12, STR1VE n=7) at Month 10. Data reported as of March 8, 2019.
CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders.

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Motor Function Improvements With Onasemnogene Abeparvovec

	START (Cohort 2, n=12)	STR1VE (N=22)[a]
Median exposure to treatment	24 months	10.1 months
Achieved CHOP INTEND score, n (%)		
≥40 points	11 (92)	21 (95)
≥50 points	11 (92)	11 (50)
≥60 points	4 (33)	2 (9)

94% with score ≥40

In START and STR1VE, 32 of 34 patients have reached a CHOP INTEND score ≥40 points

START has completed. STR1VE is ongoing.

[a]Data reported as of March 8, 2019.

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Motor Milestones With Onasemnogene Abeparvovec

Motor Milestones Reached, n (%)	START (Cohort 2, n=12)[1,2]	STR1VE (N=22)[a]
Head control[b]	11 (92)	16/20 (80)[c]
Rolls from back to sides[d]	9 (75)	9 (41)
Sitting without assistance		
≥10 seconds[e]	10 (83)	9 (41)
≥30 seconds[f]	9 (75)	11 (50)
Stands with assistance[g]	2 (17)	1 (5)
Pulls to stand[h]	2 (17)	1 (5)
Median exposure to treatment, months	24	10.1
Mean (median) age at last visit, months	27.9 (27.8)	13.8 (14.4)
Patients ≥12 months, n (%)[i]	12 (100)	15 (68)

START has completed. STR1VE is ongoing.

[a]Data reported as of March 8, 2019; [b]Bayley-III, gross motor subtest item #4 (holds head erect ≥3 seconds without support). [c]Two patients reached the milestone of head control at the first screening visit (prior to onasemnogene abeparvovec dosing), therefore total n=20 was used for the STR1VE calculation. [d]Bayley-III, gross motor subtest item #20. [e]In accordance with the WHO-MGRS criteria. [f]Bayley-III, gross motor subtest item #26. [g]Bayley-III, gross motor subtest item #33 (supports own weight for ≥2 seconds). [h]Bayley-III, gross motor subtest item #35 (raises self to standing position using chair or other convenient object for support). [i]At last visit.

Bayley-III, Bayley Scales of Infant and Toddler Development, Version 3.

1. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–1722. 2. Lowes LP, et al. *Pediatric Neurology*. 2019. doi: 10.1016/j.pediatrneurol.2019.05.005.

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Safety and Tolerability of Onasemnogene Abeparvovec in STR1VE and START: TEAEs

Safety Overview		
Patients, n (%)	**START (N=15)**	**STR1VE[a] (N=22)**
≥1 TEAE (any grade)	15 (100)	22 (100)
Grade ≥3 TEAEs	13 (86.7)	10 (45.5)
Treatment-related TEAEs[b]	4 (26.7)	12 (54.5)
Serious TEAE[d]	13 (86.7)	10 (45.5)

TEAEs of Special Interest		
Patients, n (%)	**START (N=15)**	**STR1VE[a] (N=22)**
≥1 TEAE of special interest	4 (26.7)	8 (36.4)
Liver-related investigations, signs and symptoms (SMQ)	4 (26.7)	7 (31.8)
Thrombocytopenia (SMQ)	0	3 (13.6)

START has completed. STR1VE is ongoing.

[a]Data reported as of March 8, 2019; [b]Adverse events were considered related to treatment if the event was classified as possibly, probably, or definitely related to study treatment.
TEAE, treatment-emergent adverse event.

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Time to Treatment Effect Summary

- Onasemnogene abeparvovec in START and STR1VE appears to induce a rapid improvement in motor function, as measured by CHOP INTEND scores
- Early diagnosis and treatment is critical to maximize clinical improvements in these patients

Next we will discuss onasemnogene abeparvovec vs natural history

CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; SMA1, spinal muscular atrophy type 1.
1. Data from the PNCR database. 2. Kolb SJ, et al. *Ann Neurol.* 2017;82:883–891. 3. Mendell JR, et al. *N Engl J Med.* 2017;377:1713–1722.

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Methods

Objective

- To describe the impact of treatment advancements in patients with SMA1 and contextualize them in light of the dire natural history, absent treatment

Patient Matching

- For this analysis, patients in the PNCR study[1] and NeuroNEXT SMA Infant Biomarker study (NN101; NCT01736553)[2] were selected on the basis of the following:
  - Age of onset ≤6 months
  - Biallelic deletion of *SMN1* (exon 7/8 common homozygous deletion)
  - Two copies of *SMN2*

NN101, NeuroNEXT SMA Infant Biomarker study; PNCR, Pediatric Neuromuscular Clinical Research; SMA1, spinal muscular atrophy type 1; *SMN1*, survival motor neuron 1; *SMN2*, survival motor neuron 2.
1. Finkel RS, et al. *Neurology.* 2014;83:810–817. 2. Kolb SJ, et al. *Ann Neurol.* 2017;82:883–891.

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Key Clinical Trial Characteristics

	PNCR[1,a]	NeuroNEXT (NN101; NCT01736553)[2,a]	Onasemnogene Abeparvovec	
			START (CL-101; NCT02122952)[3,4]	STR1VE (CL-303; NCT03306277)[5]
Phase	N/A	N/A	1/2a	3
Design	Longitudinal natural history	Longitudinal natural history	Single arm, open label	Single arm, open label
Care/Treatment	Best supportive care	Best supportive care	Onasemnogene abeparvovec	
Last (end-of-trial) visit	Up to 36 months	Up to 24 months	24 months	18 months of age
Age eligible for study enrollment	N/A	<6 months	<6 months[b]	<6 months
Patients included in the analysis	23	16	12 (high dose)	22

START has completed. STR1VE is ongoing.

[a]PNCR and NN101: Patients with age of onset ≤6 months, biallelic deletion of *SMN1* (exon 7/8 common homozygous deletion) and 2 copies of *SMN2* were included in this analysis. [b]At the time of treatment, one patient was 7.9 months old.

N/A, not available; NN101, NeuroNEXT SMA Infant Biomarker study; PNCR, Pediatric Neuromuscular Clinical Research; *SMN1*, survival motor neuron 1; *SMN2*, survival motor neuron 2.

1. Finkel RS, et al. *Neurology*. 2014;83:810–817. 2. Kolb SJ, et al. *Ann Neurol*. 2017;82:883–891. 3. Mendell JR, et al. *N Engl J Med*. 2017;37:1713–1722. 4. Al-Zaidy SA, et al. *J Neuromuscul Dis*. 2019; doi: 10.3233/JND-190403. 5. https://clinicaltrials.gov/ct2/show/NCT03306277.

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Key Study Outcomes

	PNCR[1,2]	NN101[3-5]	Onasemnogene Abeparvovec	
			START[5,6]	STR1VE[7]
Combined survival endpoint	Death or ≥16 hours/day of continuous assistance for ≥14 days in the absence of acute, reversible illness or perioperative state	Death or tracheostomy	Death or ≥16 hours/day of continuous assistance for ≥14 days in the absence of acute, reversible illness or perioperative state	Death or tracheostomy
CHOP INTEND	Change from baseline[a]	Change from baseline	Change from baseline	Change from baseline
Nutritional support	Nasogastric or gastrostomy tube	Gastrostomy tube	Nasogastric or gastrostomy tube	Nasogastric or gastrostomy tube
Ventilatory support	Noninvasive ventilation or tracheostomy	Not reported	Noninvasive ventilation or tracheostomy	Noninvasive ventilation or tracheostomy
Motor-milestone achievements	Sitting without support, standing alone or walking alone	Sitting without support, standing alone or walking alone	Sitting unassisted, standing unassisted, and walking unassisted	Sitting unassisted, standing unassisted, and walking unassisted
Assessment tools	-	-	Bayley-III, WHO, and independent video recordings	Bayley-III, WHO, and independent video recordings

START has completed. STR1VE is ongoing.

[a]Results not shown.

Bayley-III, Bayley Scales of Infant and Toddler Development, Version 3; CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; NN101, NeuroNEXT SMA Infant Biomarker study; PNCR, Pediatric Neuromuscular Clinical Research; WHO, World Health Organization.

1. Finkel RS, et al. *Neurology*. 2014;83:810–817. 2. Data from the PNCR database. 3. Kolb SJ, et al. *Ann Neurol*. 2017;82:883–891. 4. Data from the NeuroNEXT database. 5. Al-Zaidy SA, et al. *J Neuromuscul Dis*. 2019; doi: 10.3233/JND-190403. 6. Mendell JR, et al. *N Engl J Med*. 2017;37:1713–1722. 7. https://clinicaltrials.gov/ct2/show/NCT03306277.

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Demographic and Other Baseline Characteristics

Characteristic	PNCR[a] (n=23)[1]	NN101[a] (n=16)[2]	START (n=12)[2,3]	STR1VE (N=22)
Sex, female, n (%)	12 (52.2)	8 (50.0)	7 (58.3)	12 (54.5)
Race, white, n (%)	16 (69.6)	15 (93.8)	11 (91.7)	11 (50)
Ethnicity, Hispanic, n (%)	3 (13.0)	5 (31.3)	2 (16.7)	4 (18.2)
Age at SMA onset (months) Mean (range)	3.0 (0.5 – 6.0)	N/A	1.4 (0.0–3.0)	1.9 (0–4.0)
Age at first dose (months) Mean (range)	N/A	N/A	3.4 (0.9–7.9)[b]	3.7 (0.5–5.9)
CHOP INTEND score Mean (SD)	24.6 (11.6)	20.3 (7.3)	28.2 (12.3)	32.0 (9.69)

START has completed. STR1VE is ongoing.

[a]PNCR and NN101: Patients with age of onset ≤6 months, biallelic deletion of *SMN1* (exon 7/8 common homozygous deletion) and 2 copies of *SMN2* were included in this analysis. [b]At the time of treatment, one patient was 7.9 months old.

CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; N/A, not available; NN101, NeuroNEXT SMA Infant Biomarker study; PNCR, Pediatric Neuromuscular Clinical Research; SD, standard deviation; SMA, spinal muscular atrophy; *SMN1*, survival motor neuron 1; *SMN2*, survival motor neuron 2.

1. Data from the PNCR database. 2. Al-Zaidy SA, et al. *J Neuromuscul Dis*. 2019; doi: 10.3233/JND-190403. 3. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–1722.

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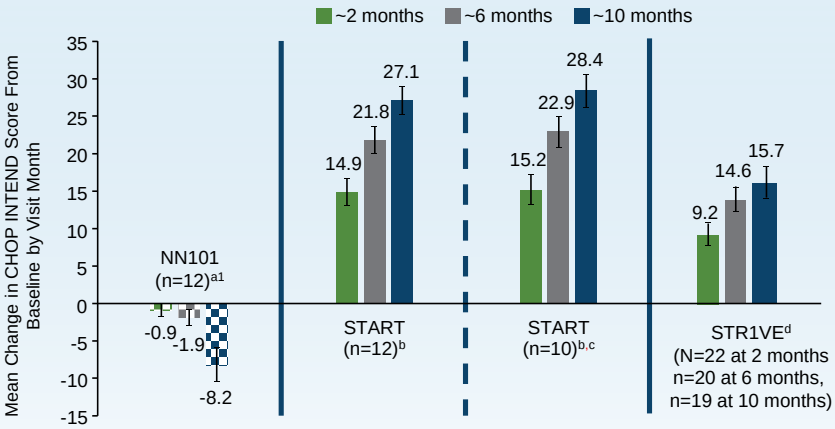
Treatment Landscape for SMA1 Patients: Motor Function – Mean Change in CHOP INTEND Scores



- Mean CHOP INTEND scores decreased with best supportive care in natural history[1]

Sensitivity Analysis

- Two onasemnogene abeparvovec–treated patients with high baseline CHOP INTEND scores were excluded from the cohort (n=10)
  - Mean baseline CHOP INTEND score was 24.1 for onasemnogene abeparvovec–treated patients included in the sensitivity analysis (n=10)

START has completed. STR1VE is ongoing.

[a]NN101: CHOP INTEND at 3, 6, and 9 months; [b]Calculated least square mean; [c]Sensitivity analysis. [d]Data reported as of March 8, 2019

CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; NN101, NeuroNEXT SMA Infant Biomarker study; SMA1, spinal muscular atrophy type 1.

1. Data from the NeuroNEXT database.

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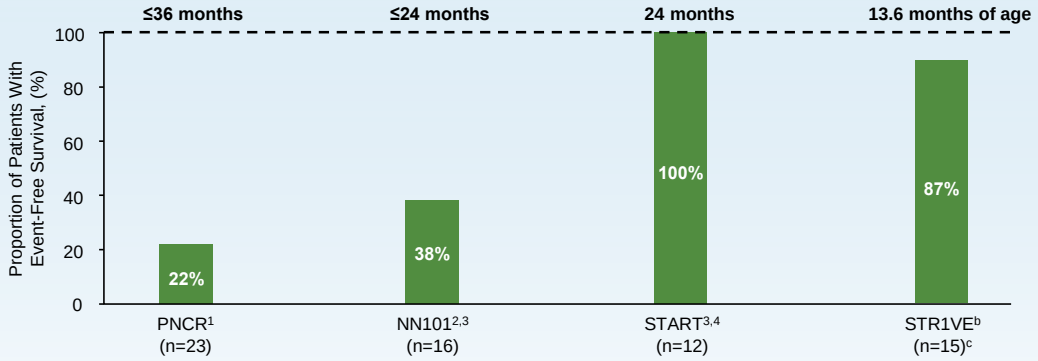
Improved Event-Free Survival With Treatment

Proportion of Patients Surviving Event-Free[a]



START has completed. STR1VE is ongoing.

[a]Proportions represent % surviving at last (end-of-trial) visit. Combined survival endpoint: PNCR, death or ≥16 hours/day of continuous assistance for ≥14 days in the absence of acute, reversible illness or perioperative state; NN101, death or tracheostomy; START, death or ≥16 hours/day of continuous assistance for ≥14 days in the absence of acute, reversible illness or perioperative state; STR1VE, death or tracheostomy; [b]Data reported as of March 8, 2019; [c]13 of 15 patients (87%) who had reached 13.6 months of age or discontinued the study prior to 13.6 months were surviving without permanent ventilation.

NN101, NeuroNEXT SMA Infant Biomarker study; PNCR, Pediatric Neuromuscular Clinical Research.

1. Data from the PNCR database. 2. Data from the NeuroNEXT database. 3. Al-Zaidy SA, et al. *J Neuromuscul Dis*. 2019; doi: 10.3233/JND-190403. 4. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–1722.

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Motor Milestone Achievements

Motor Milestone	PNCR (n=23)[1]	NN101 (n=16)[2,3]	START (n=12)[3,4]	STR1VE (N=22)[a]
Sitting without support ≥30 seconds, n (%)[b]	0	0	9 (75)	11 (50)
Stands unassisted, n (%)	0	0	2 (17)	1 (4.5)
Walks unassisted, n (%)	0	0	2 (17)	0
Last (end-of-trial) visit	**≤36 months**	**≤24 months**	**24 months**	**18 months of age**

START has completed. STR1VE is ongoing.

[a]Data reported as of March 8, 2019; [b]As determined using the Bayley-III Gross Motor subtest item 26 for START.

Bayley-III, Bayley Scales of Infant and Toddler Development, Version 3; NN101, NeuroNEXT SMA Infant Biomarker study; PNCR, Pediatric Neuromuscular Clinical Research.

1. Data from the PNCR database. 2. Kolb SJ, et al. *Ann Neurol*. 2017;82:883–891. 3. Al-Zaidy SA, et al. *J Neuromuscul Dis*. 2019; doi: 10.3233/JND-190403. 4. Mendell JR, et al. *N Engl J Med*. 2017;377:1713–1722.

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Limitations

- STR1VE is an ongoing study with relatively limited longitudinal follow-up, and continued assessment of milestones is being collected
- Cross-study comparison based on results from natural history studies and clinical trial of onasemnogene abeparvovec
- The small sample sizes of the clinical trial populations did not allow for adjustments for potential confounding factors

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Summary

- Onasemnogene abeparvovec in START and STR1VE appears to induce a rapid improvement in motor function, as measured by CHOP INTEND scores
- Patients with SMA1 receiving no disease-modifying therapy have low survival and rapid motor function decline[1,2]
- Onasemnogene abeparvovec when compared to natural history, improved survival and motor function outcomes and showed greater event-free survival and increased CHOP INTEND scores in both START and STR1VE
- Early diagnosis may be needed and treatment is critical to maximize clinical improvements in these patients
- Long-term monitoring of patients treated with onasemnogene abeparvovec is needed to confirm maintenance of observed effects
- The findings of this study should be interpreted in the context of its limitations; nevertheless, the results are based on the best available evidence to date

CHOP INTEND, Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders; SMA1, spinal muscular atrophy type 1.

1. Data from the PNCR database. 2. Kolb SJ, et al. *Ann Neurol.* 2017;82:883–891.

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Acknowledgments

- The authors wish to thank the investigators and site coordinators, and most importantly, the patients, families, and caregivers for their participation in the START and STR1VE clinical trials
- Medical writing assistance was provided by 360 Medical Writing and Ashfield Healthcare Communications, part of UDG Healthcare plc and was funded by AveXis, Inc.

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Backup

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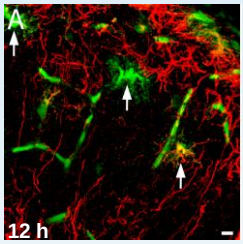
Pre-clinical Development of Onasemnogene Abeparvovec *SMN* GRT

- In the severe SMA mouse model, following a single intravenous injection of GRT
  - Transgene was rapidly expressed in astrocytes and motor neurons



A) At 12 hours post-injection of scAAV9 CB GFP, GFP (green) was detected in astrocytes (red, GFAP)

Arrows, GFP-GFAP co-localization
Scale bars, A) 10 µm

AAV9, adeno-associated virus serotype 9; CB, chicken β-actin; GFAP, glial fibrillary acidic protein; GFP, green fluorescent protein; GRT, gene-replacement therapy; sc, self-complimentary; SMA1, spinal muscular atrophy type 1; *SMN*, survival motor neuron gene.

1. Foust KD, et al. Poster presented at: American Society of Gene & Cell Therapy (ASGCT) 20th Annual Meeting; May 10–13, 2017; Washington DC. 2. Mulcahy PJ, et al. *Hum Gene Ther*. 2014;25:575–586.

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Pre-clinical Development of Onasemnogene Abeparvovec *SMN* GRT

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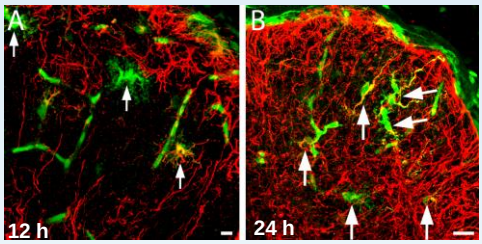
  - Transgene was rapidly expressed in astrocytes and motor neurons



A) At 12 hours post-injection of scAAV9 CB GFP, GFP (green) was detected in astrocytes (red, GFAP)
B) GFP (green) labeling of astrocytes (red, GFAP) increased at 24 hours post-injection

Arrows, GFP-GFAP co-localization
Scale bars, A) 10 µm and B) 20 µm.

AAV9, adeno-associated virus serotype 9; CB, chicken β-actin; GFAP, glial fibrillary acidic protein; GFP, green fluorescent protein; GRT, gene-replacement therapy; sc, self-complimentary; SMA1, spinal muscular atrophy type 1; *SMN*, survival motor neuron gene.

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Pre-clinical Development of Onasemnogene Abeparvovec *SMN* GRT

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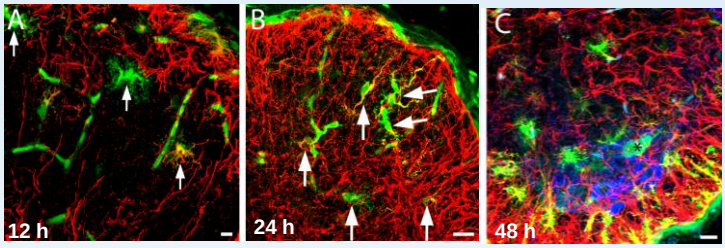
  - Transgene was rapidly expressed in astrocytes and motor neurons



A) At 12 hours post-injection of scAAV9 CB GFP, GFP (green) was detected in astrocytes (red, GFAP)
B) GFP (green) labeling of astrocytes (red, GFAP) increased at 24 hours post-injection
C) GFP (green) in motor neurons (blue, ChAT) was detected at 48 hours post-injection
Arrows, GFP-GFAP co-localization; asterisk, GFP-ChAT co-localization.
Scale bars, A) 10 µm and B-C) 20 µm.

AAV9, adeno-associated virus serotype 9; CB, chicken β-actin; ChAT, choline acetyltransferase; GFAP, glial fibrillary acidic protein; GFP, green fluorescent protein; GRT, gene-replacement therapy; sc, self-complimentary; SMA1, spinal muscular atrophy type 1; *SMN*, survival motor neuron gene.

1. Foust KD, et al. Poster presented at: American Society of Gene & Cell Therapy (ASGCT) 20th Annual Meeting; May 10–13, 2017; Washington DC. 2. Mulcahy PJ, et al. *Hum Gene Ther*. 2014;25:575–586.

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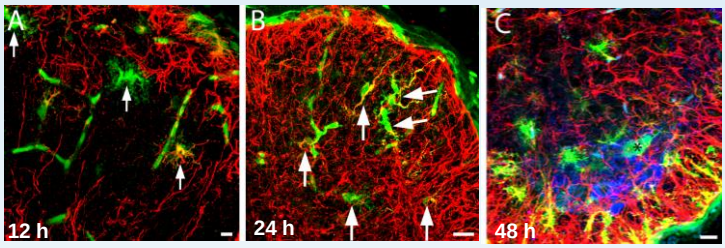
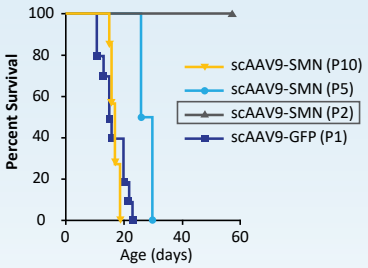
Pre-clinical Development of Onasemnogene Abeparvovec *SMN* GRT

- In the severe SMA mouse model, following a single intravenous injection of GRT
  - Transgene was rapidly expressed in astrocytes and motor neurons
  - Animals treated earlier had greater survival; median survival extended from ~15.5 days to >250 days[1,2]





A) At 12 hours post-injection of scAAV9 CB GFP, GFP (green) was detected in astrocytes (red, GFAP)
B) GFP (green) labeling of astrocytes (red, GFAP) increased at 24 hours post-injection
C) GFP (green) in motor neurons (blue, ChAT) was detected at 48 hours post-injection
Arrows, GFP-GFAP co-localization; asterisk, GFP-ChAT co-localization.
Scale bars, A) 10 µm and B-C) 20 µm.

AAV9, adeno-associated virus serotype 9; CB, chicken β-actin; ChAT, choline acetyltransferase; GFAP, glial fibrillary acidic protein; GFP, green fluorescent protein; GRT, gene-replacement therapy; P, post-natal day; sc, self-complimentary; SMA1, spinal muscular atrophy type 1; *SMN*, survival motor neuron gene.

1. Foust KD, et al. Poster presented at: American Society of Gene & Cell Therapy (ASGCT) 20th Annual Meeting; May 10–13, 2017; Washington DC. 2. Mulcahy PJ, et al. *Hum Gene Ther*. 2014;25:575–586.

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