Delandistrogene Moxeparvovec in Duchenne Muscular Dystrophy: Long-Term EMBARK 2-Year Functional Outcomes, Safety, and Micro-Dystrophin Expression

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Background

- Delandistrogene moxeparvovec is an rAAVrh74 vector-based gene transfer therapy that delivers a transgene encoding delandistrogene moxeparvovec micro-dystrophin, an engineered, functional form of dystrophin shown to stabilize or slow disease progression in ambulatory patients with DMD (**Supplementary Figure 1**)[1–4]
- Delandistrogene moxeparvovec is approved in the USA and in other select countries[5–13]

Methods

- In the absence of a placebo arm, due to the crossover study design (**Supplementary Figure 2**), 2-year data of EMBARK Part 1-treated patients and post hoc 1-year follow-up data of patients treated at 8–9 years of age were compared with an EC cohort of patients with DMD using propensity-score weighting[14–16]*
- Patients receiving only corticosteroid regimens from the FOR-DMD, BioMarin PRO-DMD-01, and CINRG DNHS studies were included (**Table 1**)[17–20]
- Propensity-score weighting* was based on baseline:† Age, NSAA total score, TTR, 10MWR, height, weight, and BMI

*Inverse probability of treatment weighting. †Propensity-score weighting involves taking an EC group with similar age and function, but unequal distribution, and ensuring overlap after propensity-score weighting.

Objectives

- We present 2-year functional outcomes from patients treated with delandistrogene moxeparvovec in Part 1 of EMBARK (**Supplementary Table 1**) compared with a well-matched EC cohort in a prespecified analysis, Week 64 delandistrogene moxeparvovec micro-dystrophin expression and sarcolemmal localization, and 2-year safety outcomes
- We also report post hoc 1-year functional outcomes from 8–9-year-old patients treated in Part 2 vs. EC and patients with screening TTR 3.6–5.0 seconds treated in Part 1 vs. placebo

Table 1 *External control (EC) cohort entry criteria*

EC entry criteria	EC cohort: Part 1-treated 2-year data	EC cohort: Part 2-treated 8–9-year-old 1-year post hoc data
Age, years	≥4 and <8	≥5 and <10 years*
NSAA total score, points	≥14 and ≤32	≥12 and ≤34
TTR, time in seconds	≤5.75	≤11.75
10MWR, time in seconds	≤6.85	≤10.90
Corticosteroid regimen	Stable dose of oral corticosteroids for ≥12 weeks	
Assessment values	Both baseline and at least 1 post-baseline	

*A subgroup of matched EC patients aged ≥8 years was used in the presented analysis.

Results

- Prior to propensity-score weighting, there were 155 patients in the EC cohort who met the entry criteria for comparisons with Part 1-treated 2-year data and had at least one post-baseline visit (FOR-DMD, n=89; BioMarin PRO-DMD-01, n=41; CINRG DNHS, n=25)
- Baseline characteristics were well matched between patients receiving delandistrogene moxeparvovec in EMBARK Part 1 and EC patients after propensity-score weighting (**Table 2**)

Table 2 *Demographics and baseline characteristics of patients receiving delandistrogene moxeparvovec in EMBARK Part 1 and the EC cohort*

Characteristic, Mean (min, max)	EMBARK Part 1 delandistrogene moxeparvovec (N=64)	EC cohort (N=143)	Standardized mean difference after propensity-score weighting*
Age, years	5.98 (4.07, 7.87)	6.24 (4.24, 7.99)	–0.281
NSAA total score, points	23.3 (14, 32)	23.5 (15, 32)	–0.045
TTR, time in seconds	3.51 (1.85, 5.75)	3.52 (1.90, 5.70)	–0.011
10MWR, time in seconds	4.80 (3.20, 6.85)	4.78 (3.00, 6.70)	0.034
Weight, kg	21.20 (13.5, 37.4)	22.18 (14.0, 36.0)	–0.198
Height, cm	108.65 (93.5, 127.0)	110.60 (94.9, 131.1)	–0.285
BMI, kg/m^2	17.80 (13.69, 24.92)	17.90 (13.74, 23.64)	–0.042

*Inverse probability of treatment weighting. A standardized mean difference of 0.25 (absolute value) is generally considered to indicate an adequate balance between the two groups.

- Mean (SD) delandistrogene moxeparvovec micro-dystrophin expression and sarcolemmal localization were sustained from Week 12 to Week 64 in a subset of patients (**Table 3**)
- At 2 years, Part 1-treated patients showed statistically significant and clinically meaningful functional improvement vs. the matched EC cohort (**Figures 1 and 2**), with other secondary endpoints remaining stable (**Supplementary Figure 3**)

Figure 1 *Functional outcomes at 2 years compared with the EC cohort*

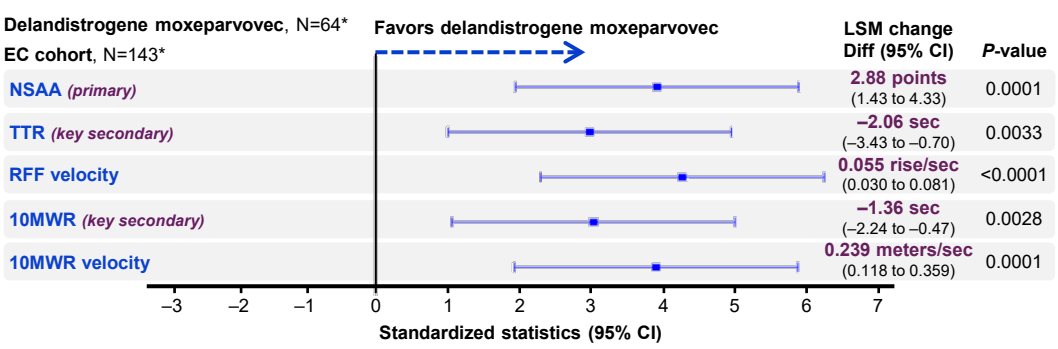


Table 3 *Delandistrogene moxeparvovec micro-dystrophin expression and sarcolemmal localization at Week 12 and Week 64*

Mean (SD)	Week 12 (n=17)	Week 64 (n=16)
Western blot, % control	34.29 (41.04)	45.68 (39.75)
PDPF, %	28.13 (26.10)	38.60 (26.93)

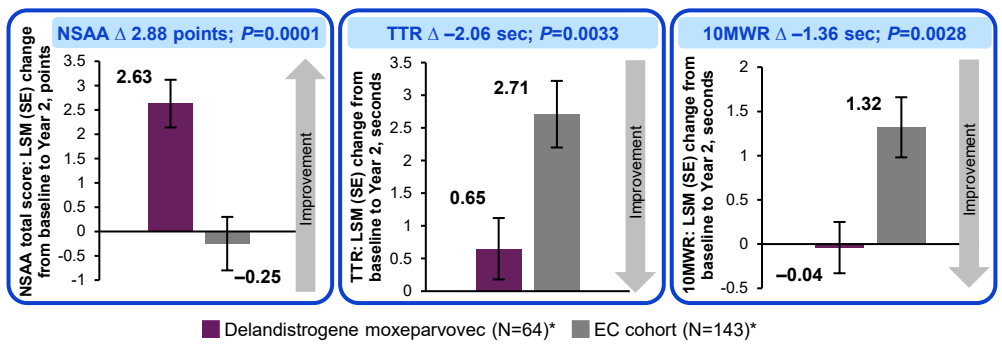
- Between Weeks 52 and 104, 15 (23.8%) patients experienced 34 TR-TEAEs (**Table 4**)
- One patient experienced two TR-SAEs of rhabdomyolysis; both resolved
- From baseline to Week 104, there were no AEs leading to study discontinuation, no clinically significant complement-mediated AEs, and no treatment-related deaths
- Safety is manageable with appropriate monitoring and treatment of AEs, which typically occur within 90 days post-infusion (**Supplementary Figure 4**)
- Outside of the EMBARK trial, to date, there have been two treatment-related deaths due to acute liver failure approximately 3 months post-infusion in non-ambulatory patients (one in the ENVISION trial and one in the commercial setting) at 15 and 16 years of age and in the advanced stage of DMD (Data on file)

Table 4 *Delandistrogene moxeparvovec safety overview*

Overview of AEs, n (%)	Baseline to Week 52 (N=63)	Weeks 52–104* (N=63)
Patients with any TEAEs	62 (98.4)	53 (84.1)
Patients with any SAEs	14 (22.2)	5 (7.9)
Patients with any TR-TEAEs	48 (76.2)	15 (23.8)
Number of TR-TEAEs	235	34
Patients with any TR-SAEs	7 (11.1)	1 (1.6)
AEs leading to study discontinuation	0 (0)	0 (0)
Treatment-related deaths	0 (0)	0 (0)

*New events between Weeks 52 and 104 (excludes ongoing events that began during Part 1 of EMBARK [baseline to Week 52]).

Figure 2 *Change from baseline to Year 2 in functional outcomes*

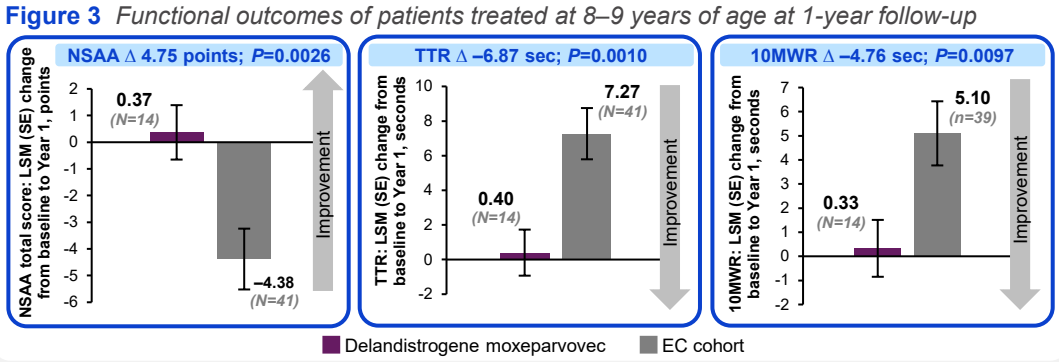


LSMs (of change from baseline) and CIs were standardized in the forest plot by dividing by the SE. Negative values for timed function tests (TTR and 10MWR) show an improvement in the time taken to achieve these endpoints. LSM differences are on original scale (without SE adjustment). Signs of timed function tests were reversed in the forest plot to align favorable directions among endpoints. Numerical results of LSM difference kept the original signs. All *P*-values reported are nominal and have not been adjusted for multiple comparisons.
*One delandistrogene moxeparvovec Part 1-treated patient did not have 2-year follow-up data. In the EC cohort, 29, 28, and 30 patients had missing Year 2 data for NSAA, TTR, and 10MWR assessments, respectively. MMRM methods account for missing data in these analyses.

Post hoc 1-year analysis of 8–9-year-olds treated in Part 2 (typically associated with the declining phase of DMD)

- Part 2 baseline characteristics were well matched between 8–9-year-old patients receiving delandistrogene moxeparvovec and EC patients after propensity-score weighting (**Supplementary Table 2**)
- At 1 year, patients treated at 8–9 years of age demonstrated statistically significant and clinically meaningful functional benefit vs. a propensity-score-weighted EC cohort (**Figure 3**)

Figure 3 *Functional outcomes of patients treated at 8–9 years of age at 1-year follow-up*

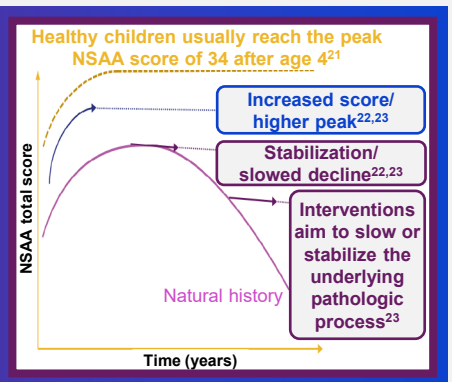


Post hoc 1-year analysis of patients with a screening TTR of 3.6–5.0 seconds treated in Part 1 (patients with some degree of functional decline [vs. those with a TTR of <3.6 seconds])

- At 1 year, treated patients with a screening TTR of 3.6–5.0 seconds demonstrated clinically meaningful and statistically significant functional benefit vs. placebo (**Supplementary Figure 5**)

Conclusions

- EMBARK Part 1-treated 2-year results indicate stabilization or slowing of disease progression compared with well-matched EC cohorts, assessed by functional outcomes prognostic for delaying loss of ambulation
- Post hoc 1-year analyses add to the totality of evidence of the delandistrogene moxeparvovec treatment effect across a broad population
- Sustained micro-dystrophin expression and sarcolemmal localization up to Week 64 demonstrate durability of the treatment effect
- Two-year safety outcomes were consistent with the EMBARK 1-year safety outcomes, with no new safety signals observed between Weeks 52 and 104



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Abbreviations

10MWR, 10-meter Walk/Run; AE, adverse event; BMI, body mass index; CI, confidence interval; CINRG, Cooperative International Neuromuscular Research Group; Diff, difference; DMD, Duchenne muscular dystrophy; DNHS, Duchenne Natural History Study; EC, external control; FOR-DMD, Finding the Optimum Regimen for DMD; LSM, least-squares mean; MMRM, mixed model for repeated measures; NSAA, North Star Ambulatory Assessment; PDPF, percent dystrophin-positive fibers; PRO-DMD, Progression of Subjects with DMD; rAAVrh74, recombinant adeno-associated virus rhesus isolate serotype 74; RFF, rise from floor; SAE, serious adverse event; SD, standard deviation; SE, standard error; TEAE, treatment-emergent adverse event; TR-SAE, treatment-related serious adverse event; TR-TEAE, treatment-related treatment-emergent adverse event; TTR, Time to Rise.

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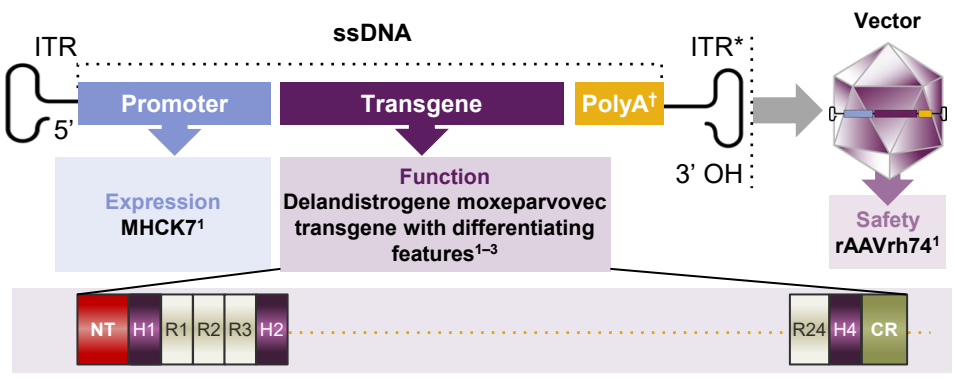
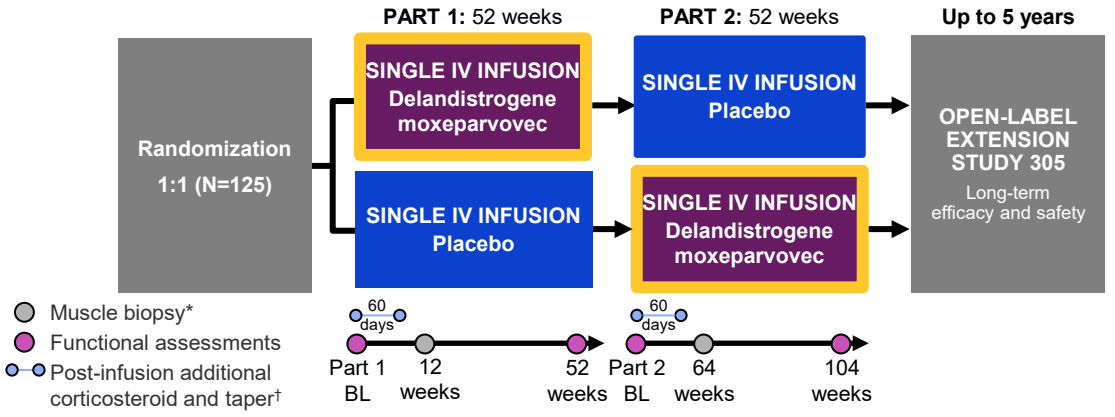
Supplementary Figure 1 *The unique delandistrogene moxeparvovec construct*



*ITRs are required for genome replication and packaging. †PolyA signals the end of the transgene to the cellular machinery that transcribes it.

Supplementary Figure 2 *EMBARK study design[4]*

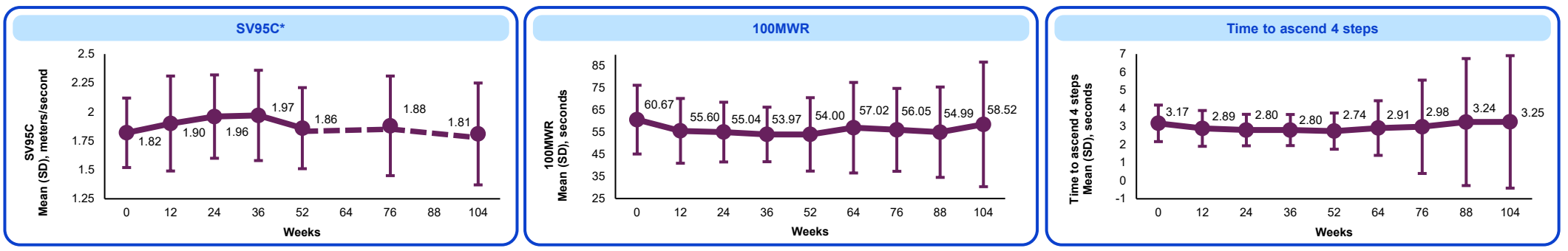


*Only a subset of patients received a muscle biopsy for expression assessments, based on site experience and feasibility. †Starting the day before infusion, patients received additional corticosteroids (prednisone 1 mg/kg/day) in addition to their baseline stable corticosteroid dose. On Day 60, the additional corticosteroid dose was tapered over a 2-week period, after which patients continued their baseline stable dose for the remainder of the study.

Supplementary Table 1 *Key inclusion criteria[4]*

Key inclusion criteria	
Ambulatory males aged ≥4 to <8 years at randomization	TTR <5 seconds at screening
Confirmed DMD diagnosis (*DMD* mutation fully contained within exons 18–79 [inclusive], excluding mutations fully contained within exon 45 [inclusive])	On a stable daily dose of oral corticosteroids for ≥12 weeks before screening
Ability to cooperate with motor assessment testing	rAAVrh74 total binding antibody titers <1:400
NSAA total score >16 and <29 points at screening	

Supplementary Figure 3 *Additional functional outcomes over 2 years*



*Data are not available for Weeks 64 and 88.

Supplementary Figure 4 *Number of patients with TR-TEAEs in time increments over 104 weeks post-infusion, listed by frequency at 0–2 weeks, n (%)**

Vomiting	31 (49.2)	3 (4.8)	0 (0)	0 (0)	0 (0)
Nausea	19 (30.2)	1 (1.6)	0 (0)	0 (0)	1 (1.6)
Decreased appetite	16 (25.4)	1 (1.6)	0 (0)	0 (0)	0 (0)
Pyrexia	10 (15.9)	0 (0)	0 (0)	0 (0)	1 (1.6)
Abdominal pain upper	7 (11.1)	1 (1.6)	0 (0)	0 (0)	0 (0)
GLDH increased†	3 (4.8)	11 (17.5)	1 (1.6)	1 (1.6)	0 (0)
Headache	2 (3.2)	0 (0)	0 (0)	0 (0)	2 (3.2)
Gamma-glutamyl transferase increase	0 (0)	5 (7.9)	0 (0)	0 (0)	1 (1.6)
Troponin-I increase	0 (0)	0 (0)	0 (0)	0 (0)	4 (6.3)
Proteinuria	0 (0)	0 (0)	0 (0)	1 (1.6)	2 (3.2)
Day 1 infusion	0–2 weeks	>2 weeks to 60 days	>60 days to 12 weeks	>12 weeks to Week 52	Week 52 to Week 104

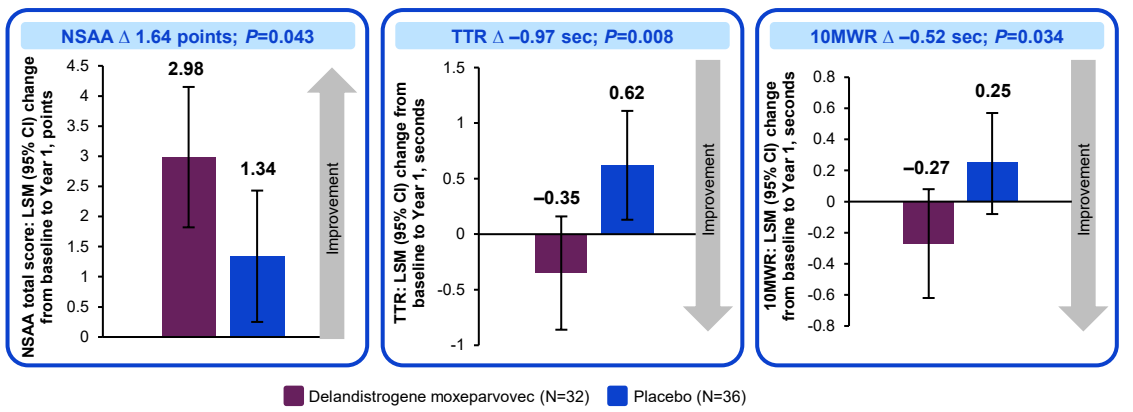
*TR-TEAEs occurring in >10% of patients in EMBARK Part 1 or in >3% of patients in Part 2. †GLDH increases were based on investigator assessment and their institution's normal range.

Supplementary Table 2 *Part 2 demographics and baseline clinical characteristics of patients treated at 8–9 years of age*

Characteristic, Mean (min, max)	EMBARK Part 2 (8–9-year-olds) delandistrogene moxeparvovec (N=14)	EC cohort (N=41)	Standardized mean difference after propensity-score weighting*
Age, years	8.51 (8.01, 9.07)	8.64 (8.00, 9.87)	–0.259
NSAA total score, points	24.4 (19, 31)	24.2 (18, 33)	0.027
TTR, time in seconds	4.22 (2.50, 8.25)	4.28 (2.90, 8.90)	–0.043
10MWR, time in seconds	5.04 (3.30, 7.10)	5.16 (3.20, 7.90)	–0.122
Weight, kg	30.80 (21.8, 49.9)	27.80 (19.4, 42.4)	0.399
Height, cm	120.52 (110.0, 131.0)	118.72 (108.8, 142.0)	0.261
BMI, kg/m^2	20.98 (15.52, 29.08)	19.59 (14.19, 25.88)	0.363

*Inverse probability of treatment weighting.

Supplementary Figure 5 *Functional outcomes of patients with screening TTR 3.6–5.0 seconds at 1-year follow-up*



All *P*-values reported are nominal and have not been adjusted for multiple comparisons.

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Abbreviations

10MWR, 10-meter Walk/Run; 100MWR, 100-meter Walk/Run; BL, baseline; BMI, body mass index; CI, confidence interval; CR, cysteine-rich domain; DMD, Duchenne muscular dystrophy; EC, external control; GLDH, glutamate dehydrogenase; H, hinge; ITR, inverted terminal repeat; IV, intravenous; LSM, least-squares mean; NSAA, North Star Ambulatory Assessment; NT, N-terminal; OH, hydroxide; polyA, polyadenylation; R, repeat; rAAVrh74, recombinant adeno-associated virus rhesus isolate serotype 74; SD, standard deviation; ssDNA, single-stranded DNA; SV95C, stride velocity 95th centile; TR-TEAE, treatment-related treatment-emergent adverse event; TTR, Time to Rise.