Long-Term Functional Outcomes and Safety Following Delandistrogene Moxeparvovec Treatment in DMD: EMBARK 2-Year Results

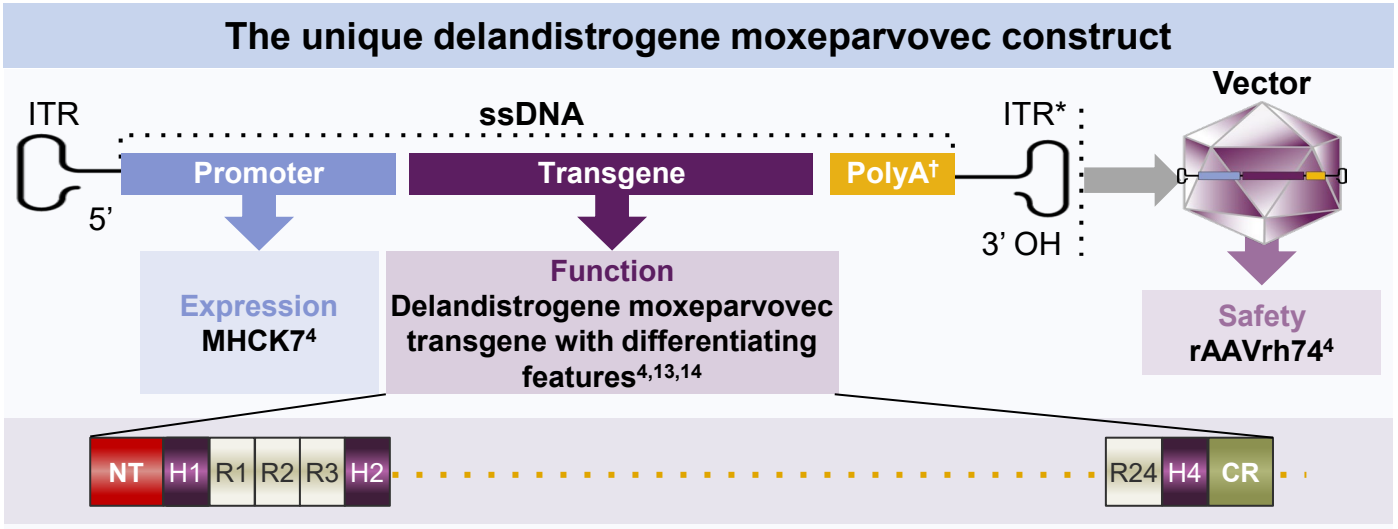
Jerry R. Mendell,[1] Francesco Muntoni,[2] Craig M. McDonald,[3] Eugenio M. Mercuri,[4] Emma Ciafaloni,[5] Hirofumi Komaki,[6] Carmen Leon-Astudillo,[7] Andrés Nascimento,[8] Crystal Proud,[9] Ulrike Schara-Schmidt,[10] Aravindhan Veerapandiyan,[11] Craig M. Zaidman,[12] Matthew Furgerson,[1] Kai Ding,[1] Preeti Singh,[1] Rachael Potter,[1] Damon R. Asher,[1] Alexander P. Murphy,[13] Carol Reid,[13] Gregory Hooper,[13] Carmen O. Torre,[13] Marianna Manfrini,[14] Jacob S. Elkins,[1] Louise R. Rodino-Klapac,[1] on behalf of the EMBARK Study Group

Author affiliations:
[1]Sarepta Therapeutics, Inc., Cambridge, MA, USA; [2]Dubowitz Neuromuscular Centre, NIHR Great Ormond Street Hospital Biomedical Research Centre, Great Ormond Street Institute of Child Health and Institute of Neurology, University College London, & Great Ormond Street Hospital Trust, London, UK; [3]UC Davis Health, Sacramento, CA, USA; [4]Pediatric Neurology Institute, Catholic University and Nemo Pediatrico, Fondazione Policlinico Gemelli IRCCS, Rome, Italy; [5]University of Rochester Medical Center, Rochester, NY, USA; [6]Translational Medical Center, National Center of Neurology and Psychiatry, Kodaira, Tokyo, Japan; [7]Department of Pediatrics, University of Florida, Gainesville, FL, USA; [8]Neuromuscular Unit, Neuropaediatrics Department, Hospital Sant Joan de Déu, Fundacion Sant Joan de Déu, CIBERER – ISC III, Barcelona, Spain; [9]Children's Hospital of the King's Daughters, Norfolk, VA, USA; [10]Department of Pediatric Neurology, Center for Neuromuscular Disorders in Children and Adolescents, University Clinic Essen, University of Duisburg-Essen, Essen, Germany; [11]Department of Pediatrics, Division of Neurology, University of Arkansas for Medical Sciences, Arkansas Children's Hospital, Little Rock, AR, USA; [12]Department of Neurology, Washington University in St Louis, St Louis, MO, USA; [13]Roche Products Ltd, Welwyn Garden City, UK; [14]F. Hoffmann-La Roche Ltd, Basel, Switzerland

Presenter

Background and objectives

- Delandistrogene moxeparvovec is an rAAVrh74 vector-based gene transfer therapy for DMD with high affinity for skeletal, respiratory, and cardiac muscles[1–4]
- Delivers a transgene encoding delandistrogene moxeparvovec micro-dystrophin[1–4]
- Approved in the USA and other select countries[5–12]



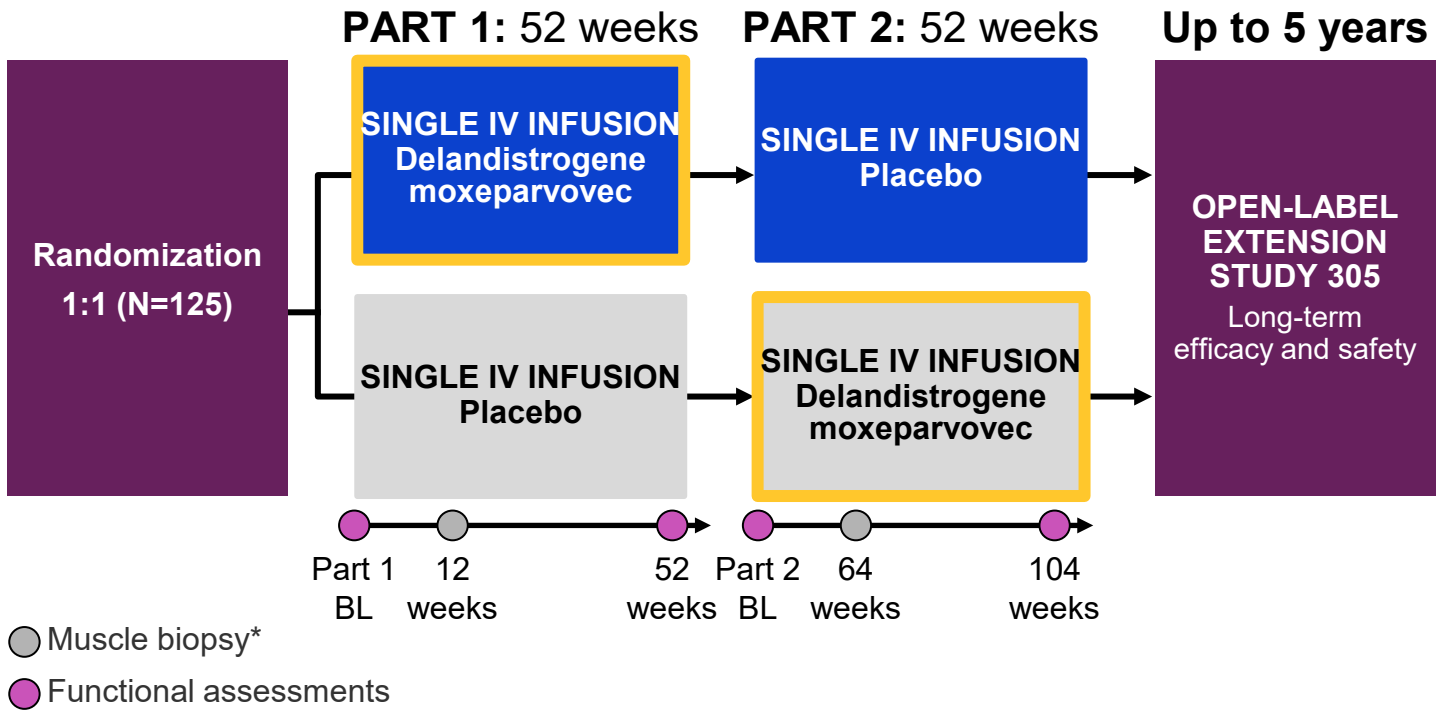
Presentation objectives

- Report **long-term, 2-year functional outcomes, safety, and micro-dystrophin expression** from patients treated in Part 1 of EMBARK[15]
- Report **post hoc 1-year functional outcomes** from **8- to 9-year-old patients (typically associated with the declining phase of DMD)** treated in Part 2 of EMBARK

*ITRs are required for genome replication and packaging. †PolyA signals the end of the transgene to the cellular machinery that transcribes it.
CR, cysteine-rich domain; DMD, Duchenne muscular dystrophy; H, hinge; ITR, inverted terminal repeat; NT, N-terminal; OH, hydroxide; polyA, polyadenylation; R, repeat; rAAVrh74, recombinant adeno-associated virus rhesus isolate serotype 74; ssDNA, single-stranded DNA.

1. Mendell JR, et al. Presented at MDA 2024; Poster #M164; 2. Asher DR, et al. *Expert Opin Biol Ther.* 2020; 20:263–274; 3. Zheng C and Baum BJ. *Methods Mol Biol*. 2008; 434:205–219; 4. Mendell JR, et al. *JAMA Neurol*. 2020; 77:1122–1131; 5. US Food and Drug Administration. ELEVIDYS® Highlights of prescribing information. https://www.fda.gov/media/169679/download (Accessed May 2025); 6. Qatar Ministry of Public Health Update, 26 July 2024. Roche data on file; 7. UAE Ministry of Health & Prevention. https://mohap.gov.ae/en/services/registered-medical-product-directory (Accessed May 2025); 8. Kuwait Ministry of Health Update, 19 February 2024. Roche data on file; 9. National Health Regulatory Authority Bahrain. Pharmacy & Pharmaceutical Products Regulation. https://www.nhra.bh/Departments/PPR/ (Accessed May 2025); 10. Ministry of Health Oman, Registration Certificate, 25 March 2024. Roche data on file; 11. Ministry of Health Israel, Registration Certificate. 27 June 2024. Roche data on file; 12. Ministry of Health Brazil. https://www.gov.br/anvisa/pt-br/assuntos/noticias-anvisa/2024/anvisa-aprova-registro-de-primeiro-produto-de-terapia-genica-para-distrofia-muscular-de-duchenne-dmd (Accessed May 2025); 13. Duan D. *Mol Ther.* 2018; 26:1–20; 14. Deng J, et al. *Front Pharmacol*. 2022; 13:950651; 15. Mendell JR, et al. Presented at MDA 2025; #P169.

EMBARK study design



Key inclusion criteria:[1]

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

Here we report:

- **2-year functional and safety outcomes and Week 64 delandistrogene moxeparvovec micro-dystrophin expression and sarcolemmal localization data from patients treated with delandistrogene moxeparvovec in Part 1 of EMBARK**
- **A post hoc analysis of 1-year functional outcomes from 8- to 9-year-old patients treated in Part 2 of EMBARK**

*Only a subset of patients received a muscle biopsy for expression assessments, based on site experience and feasibility.
BL, baseline; DMD, Duchenne muscular dystrophy; IV, intravenous; NSAA, North Star Ambulatory Assessment; rAAVrh74, recombinant adeno-associated virus rhesus isolate serotype 74; TTR, Time to Rise.
1. ClinicalTrials.gov. NCT05096221 (Accessed May 2025).

Part 1 external control (EC) cohort

In the absence of a placebo arm, due to the crossover study design, 2-year data of EMBARK Part 1-treated patients were compared with an EC cohort of patients with DMD using propensity-score weighting[1]*

Patients receiving only corticosteroid regimens from the following studies were included:

- ✓ FOR-DMD[2]
- ✓ BioMarin PRO-DMD-01[3]
- ✓ CINRG DNHS[4,5]

EC cohort entry criteria:

- ✓ Aged ≥4 and <8 years
- ✓ NSAA total score ≥14 and ≤32
- ✓ TTR ≤5.75 seconds
- ✓ 10MWR time ≤6.85 seconds
- ✓ Stable dose of oral corticosteroids for ≥12 weeks
- ✓ Had both baseline and at least one post-baseline assessment values

Propensity-score weighting* was based on baseline:†

Age	NSAA total score	TTR	10MWR
Height	Weight	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.
10MWR, 10-meter Walk/Run; BMI, body mass index; CINRG, Cooperative International Neuromuscular Research Group; DMD, Duchenne muscular dystrophy; DNHS, Duchenne Natural History Study; EC, external control; FOR-DMD, Finding the Optimum Regimen for Duchenne Muscular Dystrophy; NSAA, North Star Ambulatory Assessment; TTR, Time to Rise.

1. Mercuri E, et al. Presented at MDA 2025; #P86; 2. ClinicalTrials.gov. NCT01603407 (Accessed May 2025); 3. ClinicalTrials.gov. NCT01753804 (Accessed May 2025); 4. ClinicalTrials.gov. NCT00468832 (Accessed May 2025); 5. Spurney C, et al. *Muscle Nerve*. 2014; 50:250–256.

Part 1 demographics and baseline clinical characteristics

Baseline characteristics were well matched between patients receiving delandistrogene moxeparvovec in EMBARK Part 1 and EC patients **after propensity-score weighting***

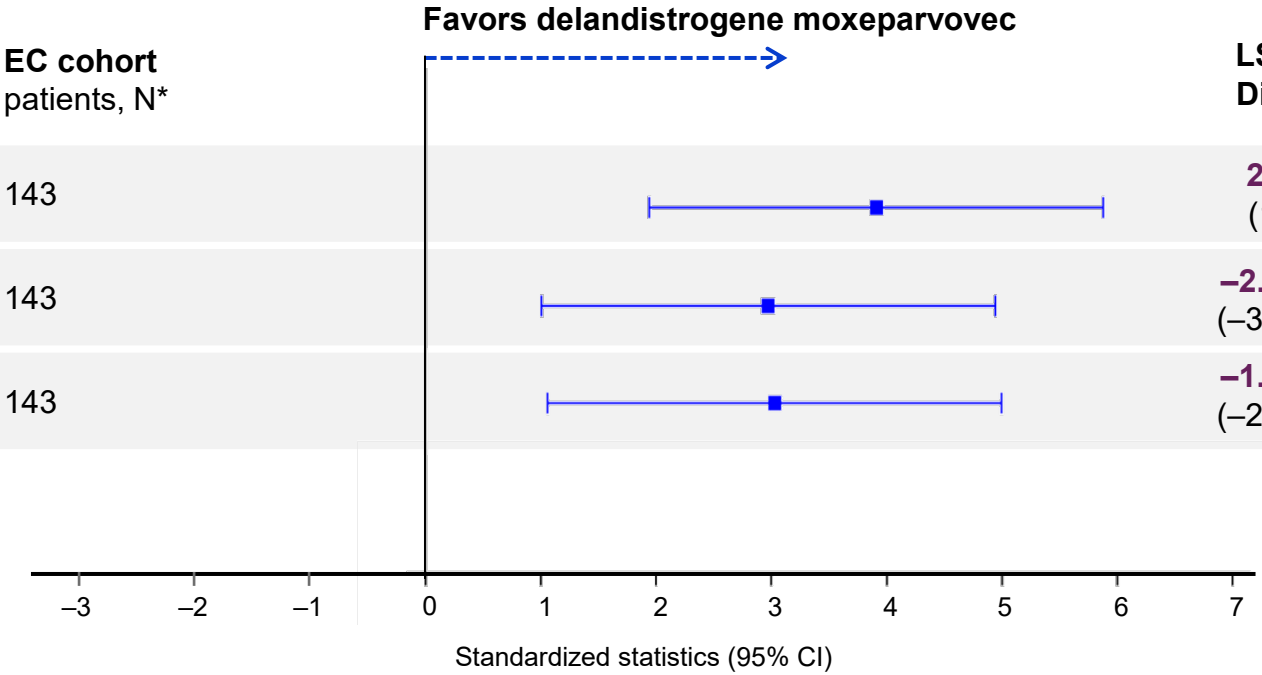
Characteristic, Mean (min, max)	EMBARK Part 1 Delandistrogene moxeparvovec (N=64)	EC cohort (N=143)†	Standardized mean difference after propensity-score weighting*
Age, years	5.98 (4.07, 7.87)	6.24 (4.24, 7.99)	–0.281
NSAA total score, points	23.3 (14, 32)	23.5 (15, 32)	–0.045
TTR, time in seconds	3.51 (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.
†Prior to propensity-score weighting, there were 155 patients in the EC cohort who met the entry criteria and had at least one post-baseline visit (FOR-DMD, n=89; BioMarin PRO-DMD-01, n=41; CINRG DNHS, n=25).
10MWR, 10-meter Walk/Run; BMI, body mass index; CINRG, Cooperative International Neuromuscular Research Group; DNHS, Duchenne Natural History Study; EC, external control; FOR-DMD, Finding the Optimum Regimen for Duchenne Muscular Dystrophy; NSAA, North Star Ambulatory Assessment; TTR, Time to Rise.

Part 1: Functional outcomes at 2 years

At 2 years, Part 1-treated patients demonstrated **statistically significant and clinically meaningful functional benefit** versus a propensity-score-weighted EC cohort

		Delandistrogene moxeparvovec patients, N*	EC cohort patients, N*	LSM change Diff (95% CI)	*P*-value
Primary endpoint	**NSAA**	64	143	**2.88 points** (1.43–4.33)	0.0001
Key secondary endpoints	**TTR**	64	143	**–2.06 seconds** (–3.43 to –0.70)	0.0033
	10MWR	64	143	**–1.36 seconds** (–2.24 to –0.47)	0.0028



*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.

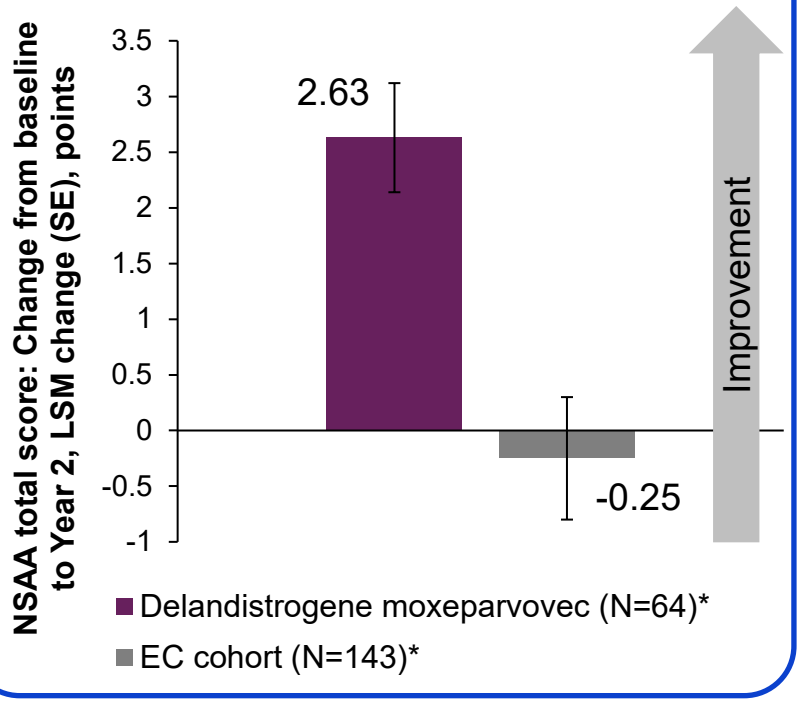
LSMs (of change from baseline) and CIs were standardized by dividing by the SE. Negative values for timed function tests (TTR and 10MWR) show an improvement in the time taken to achieve these endpoints. LSM differences are on original scale (without SE adjustment). Signs of timed function tests were 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.

10MWR, 10-meter Walk/Run; CI, confidence interval; Diff, difference; EC, external control; LSM, least-squares mean; MMRM, mixed model for repeated measures; NSAA, North Star Ambulatory Assessment; SE, standard error; TTR, Time to Rise.

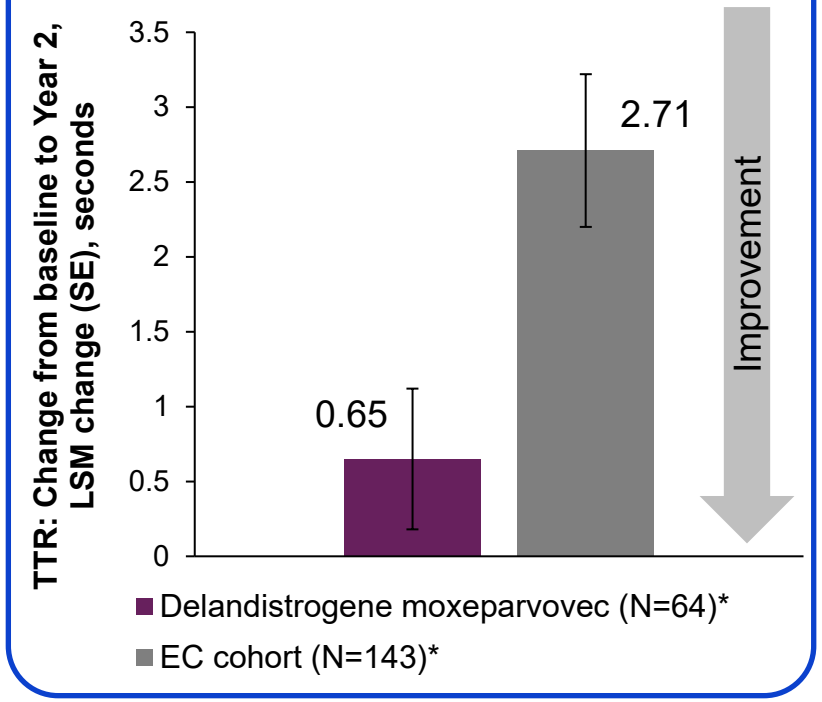
Part 1: Functional outcomes at 2 years

At 2 years, Part 1-treated patients demonstrated **statistically significant and clinically meaningful functional benefit** versus a propensity-score-weighted EC cohort

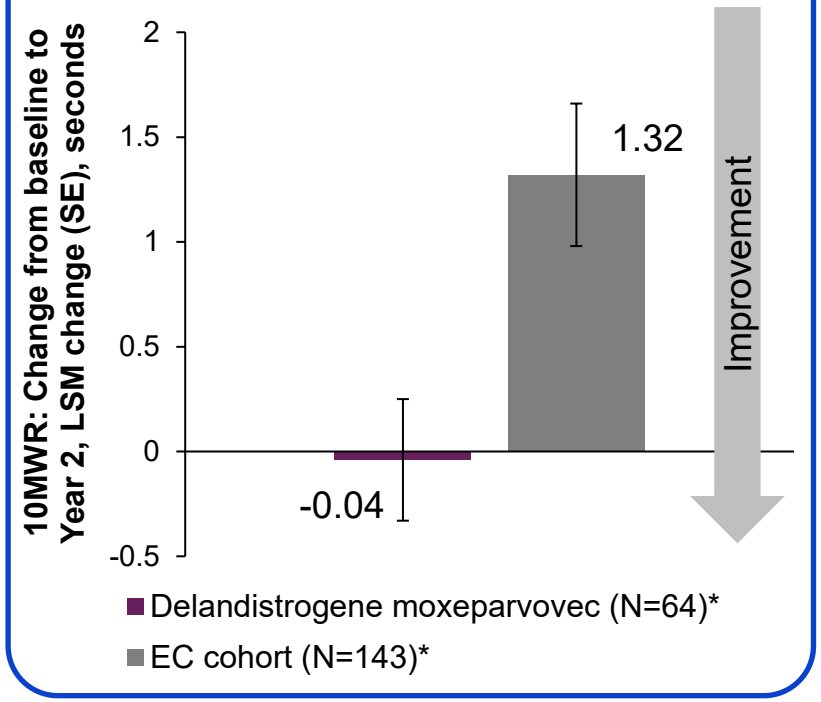
NSAA Δ 2.88 points; *P*=0.0001



TTR Δ –2.06 s; *P*=0.0033



10MWR Δ –1.36 s; *P*=0.0028

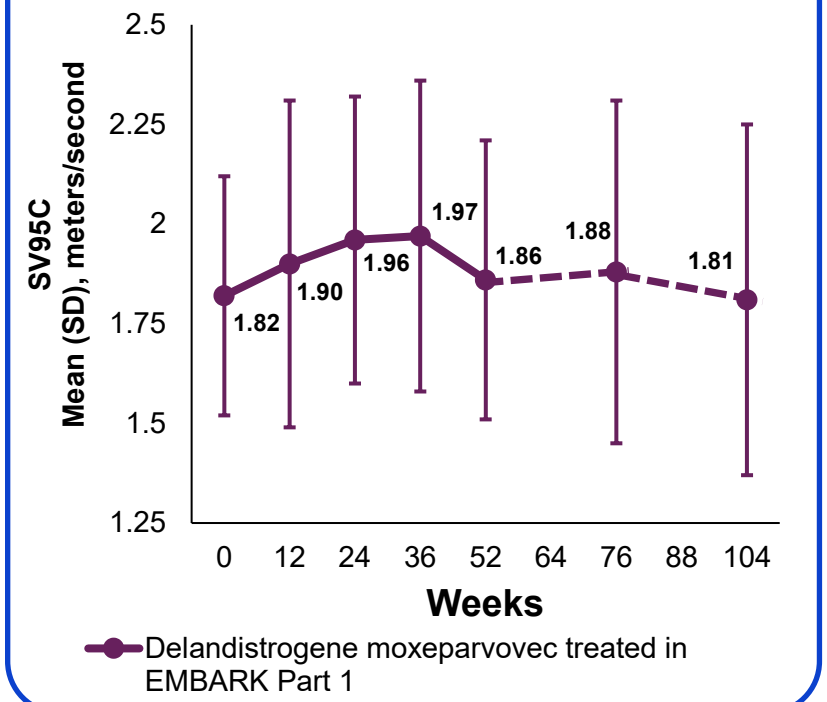


*One delandistrogene moxeparvovec Part 1-treated patient did not have 2-year follow-up data. In the EC cohort, 29, 28, and 30 patients had missing Year 2 data for NSAA, TTR, and 10MWR assessments, respectively. MMRM methods account for missing data in these analyses.
All *P*-values reported are nominal and have not been adjusted for multiple comparisons.
10MWR, 10-meter Walk/Run; EC, external control; LSM, least-squares mean; MMRM, mixed model for repeated measures; NSAA, North Star Ambulatory Assessment; SE, standard error; TTR, Time to Rise.

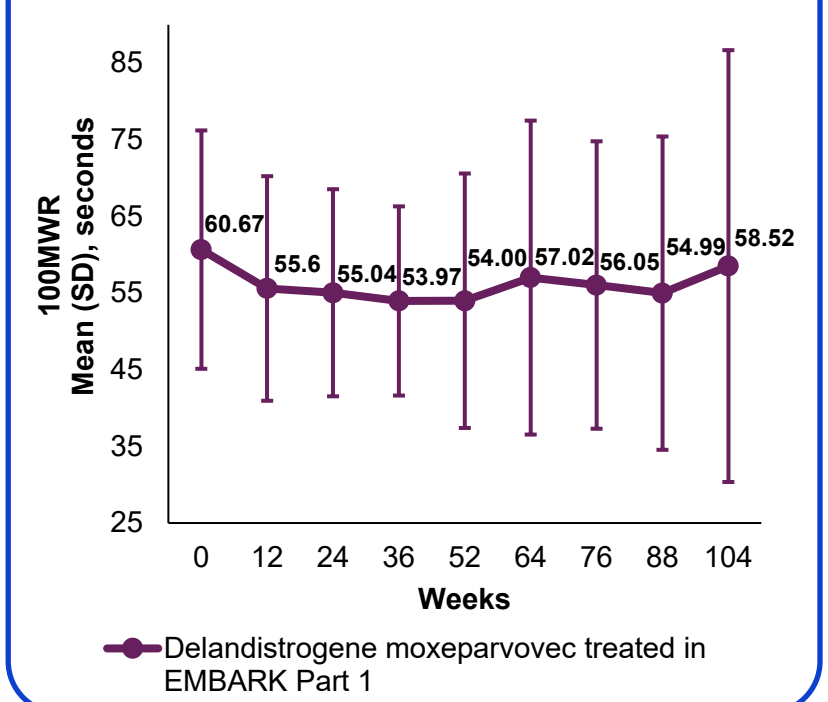
Part 1: Additional functional outcomes at 2 years

Other secondary endpoints were **stable at Year 2** in Part 1-treated patients

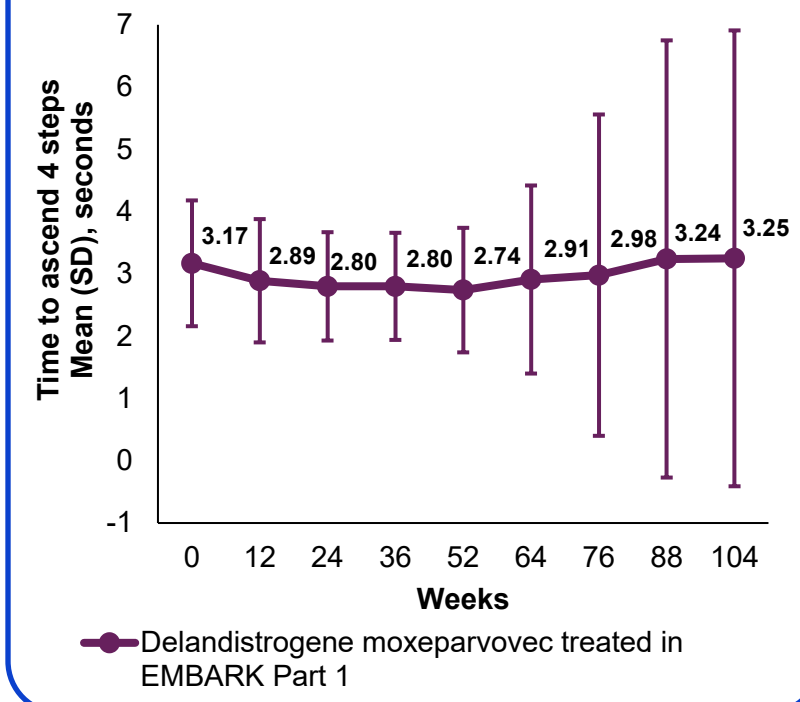
SV95C*



100MWR



Time to ascend 4 steps



*Data are not available for Weeks 64 and 88.
100MWR, 100-meter Walk/Run; SD, standard deviation; SV95C, stride velocity 95th centile.

Part 1: Delandistrogene moxeparvovec micro-dystrophin expression and sarcolemmal localization up to Week 64

Delandistrogene moxeparvovec micro-dystrophin expression and sarcolemmal localization were **sustained from Week 12 to Week 64** in a subset of patients treated in EMBARK Part 1*

Mean (SD)	Delandistrogene moxeparvovec	
	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)

*Only a subset of patients received a muscle biopsy for expression assessments, based on site experience and feasibility.
†One patient treated in Part 1 was transferred to a non-biopsy site at the beginning of Part 2 and a Week 64 biopsy was therefore not collected.
PDPF, percent dystrophin-positive fibers; SD, standard deviation.

Part 1: Overview of 2-year safety results

Between Weeks 52 and 104, **15 (23.8%) patients** experienced **34 TR-TEAEs**

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**

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])

AE, adverse event; SAE, serious adverse event; TEAE, treatment-emergent adverse event; TR-SAE, treatment-related serious adverse event; TR-TEAE, treatment-related treatment-emergent adverse event.

Part 1: Timeline of TR-TEAEs following treatment with delandistrogene moxeparvovec

Evidence to date shows that **most TR-TEAEs** had first onset **within the first 90 days of infusion**

Number of patients with TR-TEAEs, ranked 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.
GLDH, glutamate dehydrogenase; TR-TEAE, treatment-related treatment-emergent adverse event.

Post hoc analysis of 8- to 9-year-old patients treated with delandistrogene moxeparvovec in EMBARK Part 2

EC cohort selection for comparison with patients treated at 8–9 years of age in EMBARK Part 2

One-year follow-up data of patients treated at 8–9 years of age were compared with a well-matched EC cohort[1] of patients with DMD

Patients receiving only corticosteroid regimens from the following studies were included:

- ✓ FOR-DMD[2]
- ✓ BioMarin PRO-DMD-01[3]
- ✓ CINRG DNHS[4,5]

EC cohort entry criteria:

- ✓ Aged ≥5 and <10 years*
- ✓ NSAA total score ≥12 and ≤34
- ✓ TTR ≤11.75 seconds
- ✓ 10MWR time ≤10.90 seconds
- ✓ Stable dose of oral corticosteroids for ≥12 weeks
- ✓ Had both baseline and at least 1 post-baseline assessment values

Propensity-score weighting was based on baseline:†

Age	NSAA total score	TTR	10MWR
Height	Weight	BMI	

*A subgroup of matched EC patients aged ≥8 years was used in the presented analysis. †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.
10MWR, 10-meter Walk/Run; BMI, body mass index; CINRG, Cooperative International Neuromuscular Research Group; DMD, Duchenne muscular dystrophy; DNHS, Duchenne Natural History Study; EC, external control; FOR-DMD, Finding the Optimum Regimen for Duchenne Muscular Dystrophy; NSAA, North Star Ambulatory Assessment; TTR, Time to Rise.
1. Mercuri E, et al. Presented at MDA 2025; #P86; 2. ClinicalTrials.gov. NCT01603407 (Accessed May 2025); 3. ClinicalTrials.gov. NCT01753804 (Accessed May 2025); 4. ClinicalTrials.gov. NCT00468832 (Accessed May 2025); 5. Spurney C, et al. *Muscle Nerve*. 2014; 50:250–256.

Part 2 demographics and baseline clinical characteristics: Patients treated at 8–9 years of age

Part 2 baseline characteristics were well matched between 8- to 9-year-old patients receiving delandistrogene moxeparvovec and EC patients **after propensity-score weighting***

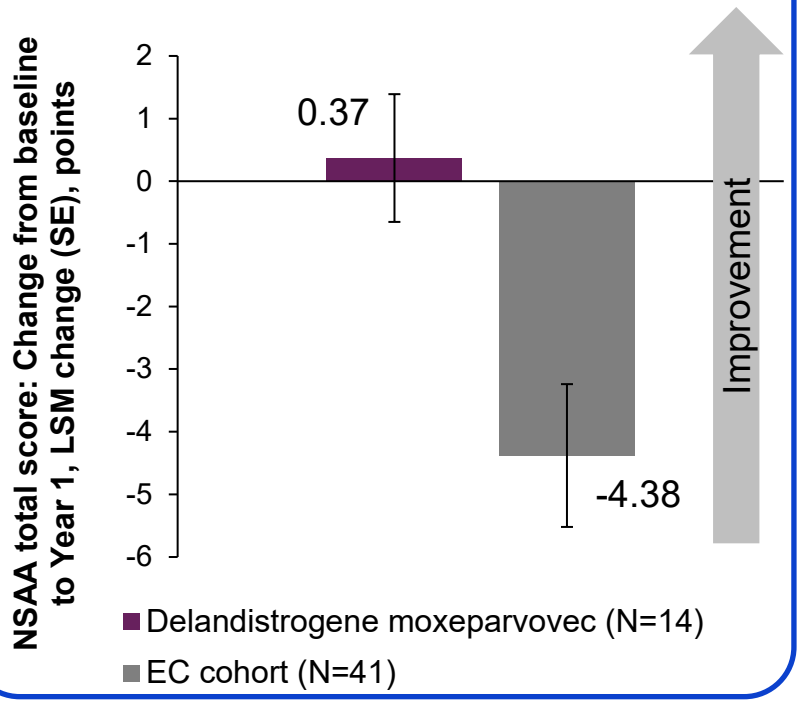
Characteristic, Mean (min, max)	EMBARK Part 2 (8- to 9-year-olds) Delandistrogene moxeparvovec (N=14)	EC cohort (N=41)	Standardized mean difference after propensity-score weighting*
Age, years	8.51 (8.01, 9.07)	8.64 (8.00, 9.87)	–0.259
NSAA total score, points	24.4 (19, 31)	24.2 (18, 33)	0.027
TTR, time 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.
10MWR, 10-meter Walk/Run; BMI, body mass index; EC, external control; NSAA, North Star Ambulatory Assessment; TTR, Time to Rise.

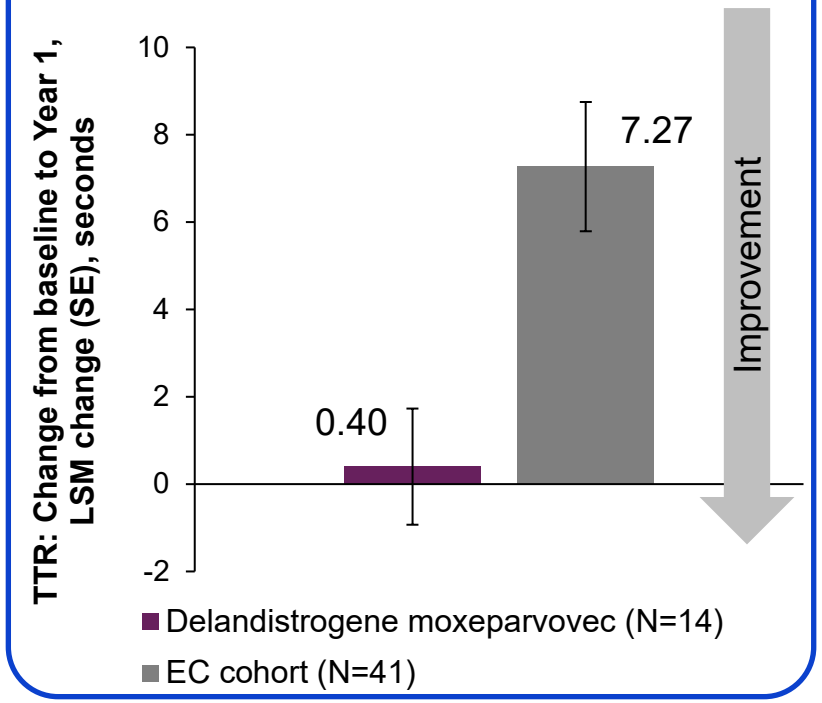
One-year follow-up: Functional outcomes of patients treated at 8–9 years of age

At 1 year post-delandistrogene moxeparvovec infusion, patients treated at 8–9 years of age demonstrated **statistically significant and clinically meaningful functional benefit** versus a propensity-score-weighted EC cohort

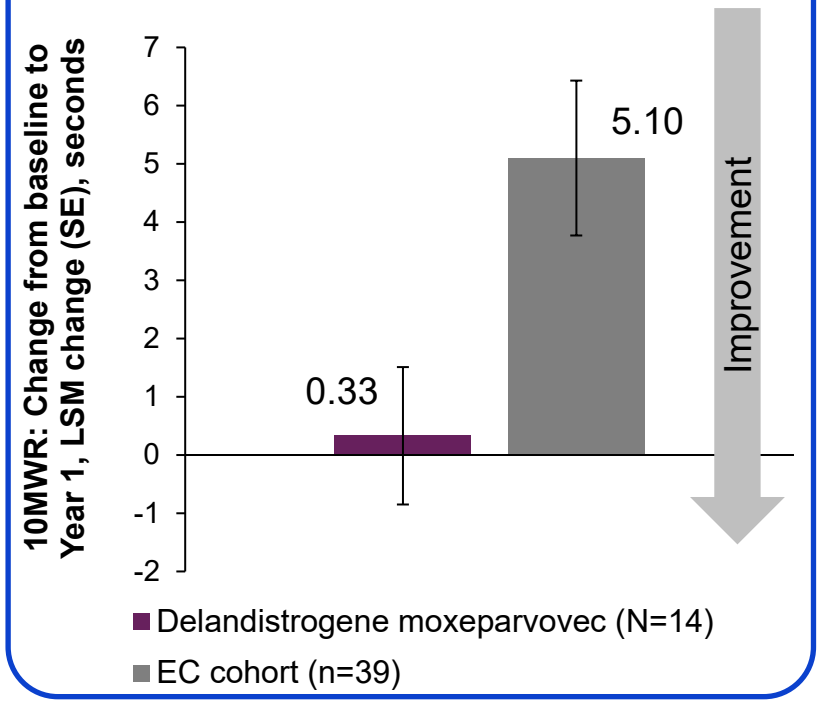
NSAA Δ 4.75 points; *P*=0.0026



TTR Δ –6.87 s; *P*=0.0010



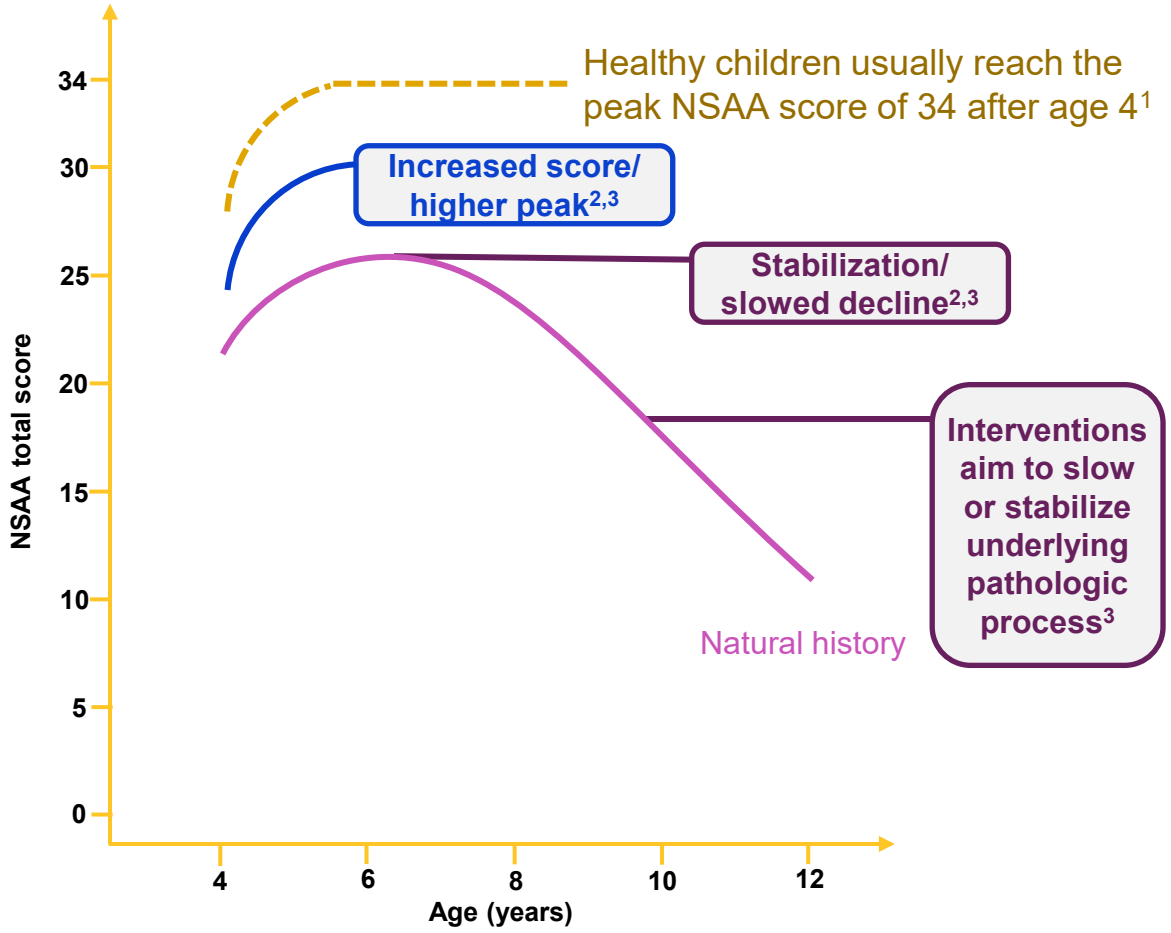
10MWR Δ –4.76 s; *P*=0.0097



All *P*-values reported are nominal and have not been adjusted for multiple comparisons.
10MWR, 10-meter Walk/Run; EC, external control; LSM, least-squares mean; NSAA, North Star Ambulatory Assessment; SE, standard error; TTR, Time to Rise.

Conclusions

- **Two-year results of patients treated at 4–7 years of age and 1-year results of patients treated at 8–9 years of age indicate stabilization or slowing of disease progression compared with well-matched EC cohorts, assessed by functional outcomes prognostic for delaying loss of ambulation**
- **Sustained micro-dystrophin expression and localization to the sarcolemma up to Week 64 demonstrate durability of the delandistrogene moxeparvovec treatment effect**
- **Two-year safety outcomes of EMBARK Part 1-treated patients were consistent with prior experience from the delandistrogene moxeparvovec clinical development program and manageable with appropriate monitoring**
  - **>800 people have received delandistrogene moxeparvovec in a clinical trial or real-world setting**



EC, external control; NSAA, North Star Ambulatory Assessment.

1. Mercuri E, et al. *PLoS One*. 2016; 11:e0160195; 2. Muntoni F, et al. *PLoS One*. 2019; 14:e0221097; 3. McDonald CM, et al. *Lancet*. 2018; 391:451–461.

EMBARK: Acknowledgements and disclosures

Acknowledgments

- The authors would like to thank the patients and their families for their participation in EMBARK, as well as the investigators and trial staff involved in EMBARK
- This study was sponsored by Sarepta Therapeutics, Inc., Cambridge, MA, USA and funded by Sarepta Therapeutics, Inc., Cambridge, MA, USA and F. Hoffmann-La Roche Ltd, Basel, Switzerland
- Medical writing and editorial support were provided by Lucretia Ramnath, PhD, of Nucleus Global, an Inizio company, in accordance with Good Publication Practice (GPP) 2022 guidelines (https://www.ismpp.org/gpp-2022) and were funded by Sarepta Therapeutics, Inc., Cambridge, MA, USA and F. Hoffmann-La Roche Ltd, Basel, Switzerland

Disclosures

- JRM received study funding from Sarepta Therapeutics, Inc. while at Nationwide Children's Hospital at the time of the EMBARK study and is currently an employee of Sarepta Therapeutics, Inc. JRM is a co-inventor of AAVrh74.MHCK7.micro-dys technology
- FM has received honoraria and grants from Sarepta Therapeutics, Inc. for participating at symposia and advisory boards and is involved as an investigator in Sarepta Therapeutics, Inc. clinical trials. He reports participation in advisory boards for Novartis, F. Hoffmann-La Roche Ltd, Edgewise Therapeutics, Dyne Therapeutics, Pfizer, PTC Therapeutics, and Italfarmaco
- CMM reports grants from Capricor Therapeutics, Catabasis, Edgewise Therapeutics, Epirium Bio, Italfarmaco, Pfizer, PTC Therapeutics, Santhera Pharmaceuticals, and Sarepta Therapeutics, Inc.; and has a consultancy/advisory role with Biomarin, Capricor Therapeutics, Catalyst, Edgewise Therapeutics, Italfarmaco, PTC Therapeutics, F. Hoffmann-La Roche Ltd, Santhera Pharmaceuticals, and Sarepta Therapeutics, Inc. He has received honoraria from PTC Therapeutics and Sarepta Therapeutics, Inc.
- EMM has received fees from AveXis, Biogen, and F. Hoffmann-La Roche Ltd
- EC has received honoraria from Sarepta Therapeutics, Inc. for participating on advisory boards and grants as an investigator in Sarepta Therapeutics, Inc. clinical trials
- HK has received grants from Sarepta Therapeutics, Inc., Pfizer, PTC Therapeutics, Taiho Pharmaceutical Co. Ltd, Chugai Pharmaceutical Co., Nippon Shinyaku Co. Ltd, and Kaneka Corporation. HK has received fees from Sarepta Therapeutics, Inc., Pfizer, PTC Therapeutics, Chugai Pharmaceutical Co., Nippon Shinyaku Co., and Kaneka Corporation
- CL-A is an investigator in Sarepta Therapeutics, Inc. clinical trials and a sub-investigator in studies sponsored by Pfizer, SolidBio, Edgewise Therapeutics, Italfarmaco, and Genentech/Roche
- AN has received fees from AveXis, Biogen, and F. Hoffmann-La Roche Ltd
- CP participates on an advisory board and is a consultant for Biogen, Sarepta Therapeutics, Inc., AveXis/Novartis Gene Therapies, Genentech/Roche, and Scholar Rock; serves as a speaker for Biogen; and is a Principal Investigator of studies sponsored by AveXis/Novartis Gene Therapies, AMO, Astellas, Biogen, CSL Behring, FibroGen, PTC Therapeutics, Pfizer, Sarepta Therapeutics, Inc., and Scholar Rock
- US-S has received honoraria for counseling and participating in invited talks from Sarepta Therapeutics, Inc. and F. Hoffmann-La Roche Ltd
- AV has a consultancy/advisory role with AMO Pharma, AveXis, Biogen, Edgewise Therapeutics, FibroGen, Novartis, Pfizer, PTC Therapeutics, Sarepta Therapeutics, Inc., UCB Pharma, Catalyst, and Scholar Rock; has received research funding from AMO Pharma, Capricor Therapeutics, Edgewise Therapeutics, FibroGen, Muscular Dystrophy Association, Novartis, Parent Project Muscular Dystrophy, Pfizer, RegenxBio, and Sarepta Therapeutics, Inc.; and has other relationship(s) with MedLink Neurology for editorial services
- CMZ has received research support from Biogen and Novartis, and has served on an advisory board for Sarepta Therapeutics
- MF, KD, PS, RP, DRA, and JSE are employees of Sarepta Therapeutics, Inc. and may have stock options
- APM, CR, GH, and COT are employees of Roche Products Ltd and may have stock options in F. Hoffmann-La Roche Ltd
- MM is an employee of F. Hoffmann-La Roche Ltd and may have stock options
- LRR-K is an employee of Sarepta Therapeutics, Inc. and may have stock options. In addition, she is a co-inventor of AAVrh74.MHCK7.micro-dys technology