Long-Term Functional Outcomes and Safety of Delandistrogene Moxeparvovec in DMD: EMBARK 2-Year and Pooled 3-Year Analyses

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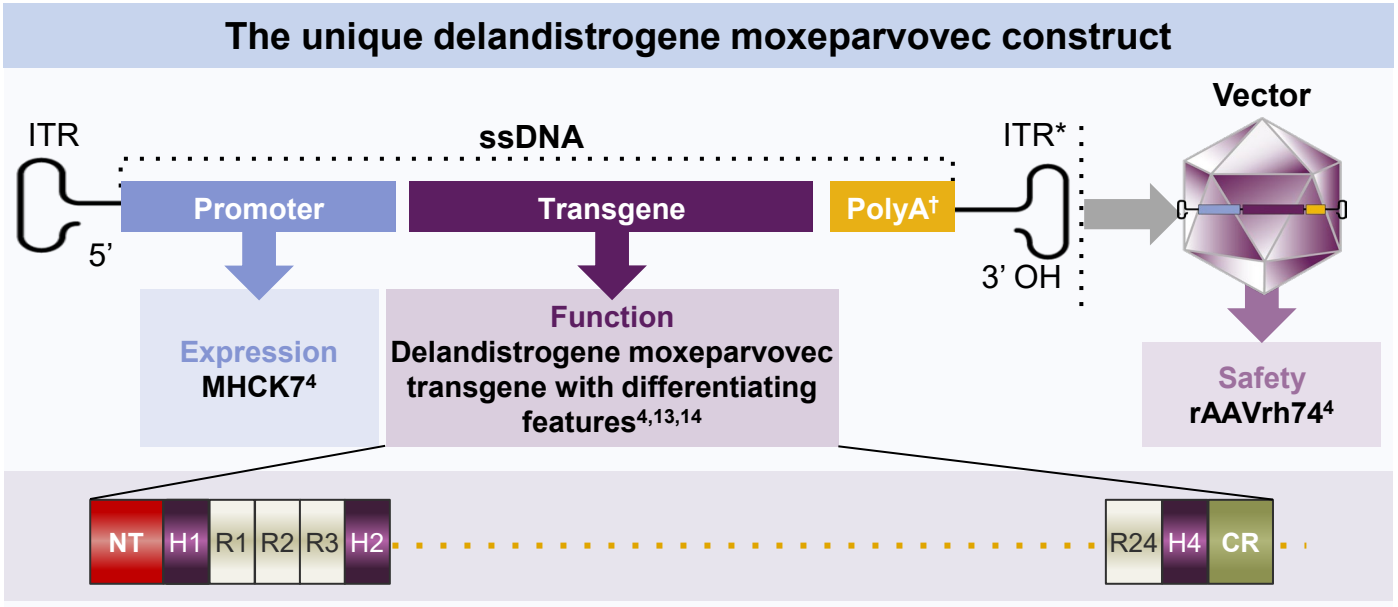
Background and objectives

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



Presentation objectives

- Report **long-term functional outcomes** from delandistrogene moxeparvovec clinical studies: **EMBARK 2-year[15]** and **pooled 3-year analyses from Study 101, Study 102 and ENDEAVOR Cohort 1[16]**
- Report **EMBARK 2-year safety**

*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; OH, hydroxide; NT, N-terminal; 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 March 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 March 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 March 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 March 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; 16. Mendell JR, et al. Presented at MDA 2025; P167.

Long-Term Functional Outcomes, Safety, and Micro-Dystrophin Expression Following Delandistrogene Moxeparvovec Treatment in DMD: EMBARK 2-Year Results (P169)

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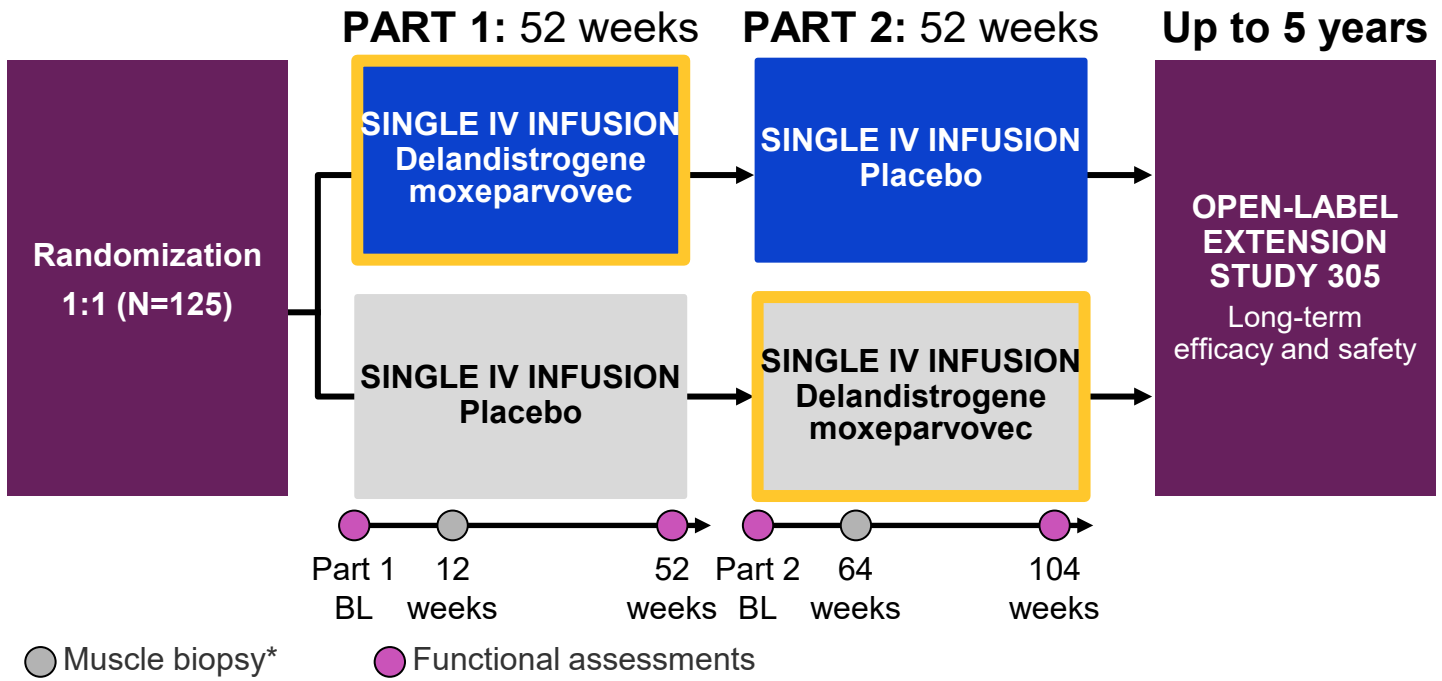
EMBARK: Study design



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

*Only a subset of patients will receive 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.
ClinicalTrials.gov. NCT05096221 (Accessed March 2025).

EMBARK: 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 1 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 March 2025); 3. ClinicalTrials.gov. NCT01753804 (Accessed March 2025); 4. ClinicalTrials.gov. NCT00468832 (Accessed March 2025); 5. Spurney C, et al. *Muscle Nerve*. 2014; 50:250–256.

EMBARK: 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***

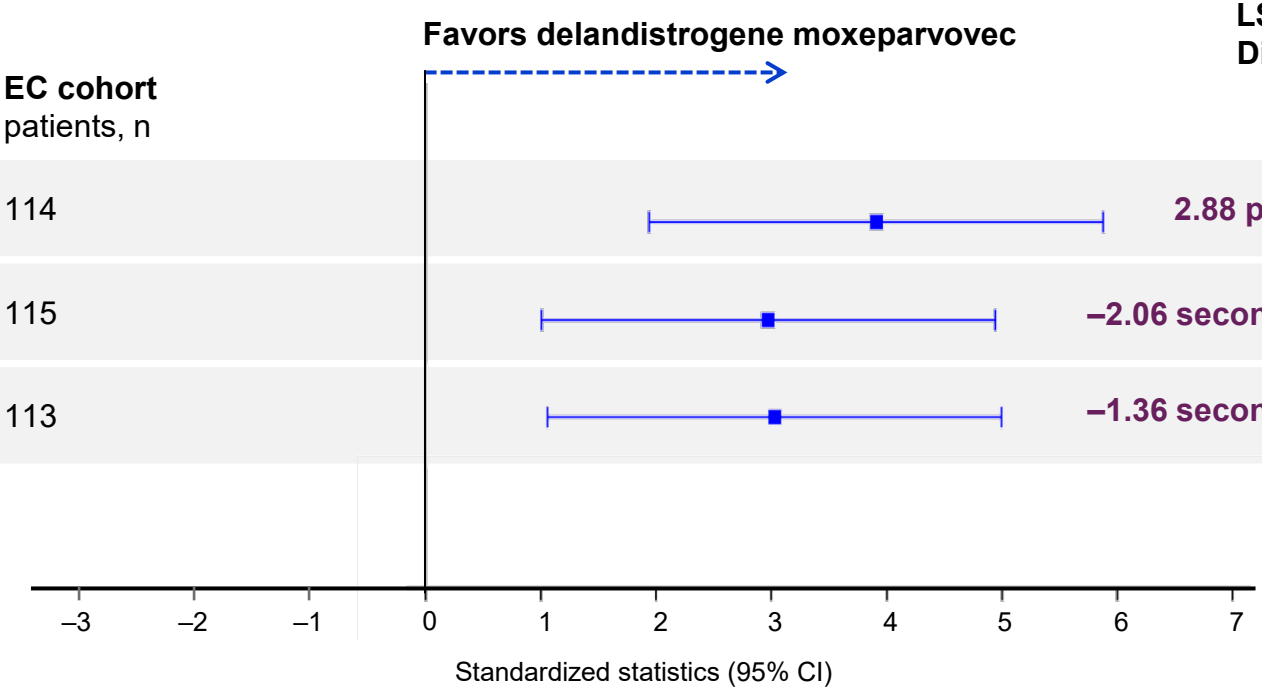
Characteristic, Mean (min, max)	EMBARK Part 1 Delandistrogene moxeparvovec (N=64)	EC cohort (N=143)†	Standardized mean difference after propensity-score weighting*
Age, years	5.98 (4.07, 7.87)	6.24 (4.24, 7.99)	–0.281
NSAA total score, points	23.3 (14, 32)	23.5 (15, 32)	–0.045
TTR, time in seconds	3.51 (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 1 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; EC, external control; NSAA, North Star Ambulatory Assessment; TTR, Time to Rise.

EMBARK 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	63	114	**2.88 points** (1.43 to 4.33)	0.0001
Key secondary endpoints	TTR	63	115	**–2.06 seconds** (–3.43 to –0.70)	0.0033
	10MWR	63	113	**–1.36 seconds** (–2.24 to –0.47)	0.0028



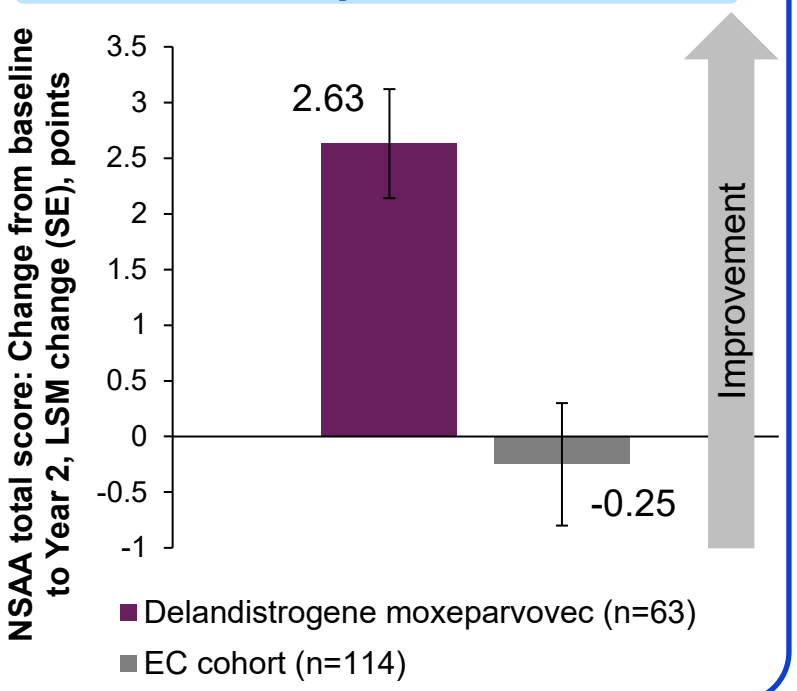
LSMs (of change from baseline) and CIs were standardized by dividing by the SE. Negative values for timed function tests (TTR and 10MWR) show an improvement in the time taken to achieve these endpoints. LSMs difference are on original scale (without SE adjustment). Signs of timed function tests were 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; NSAA, North Star Ambulatory Assessment; SE, standard error; TTR, Time to Rise.

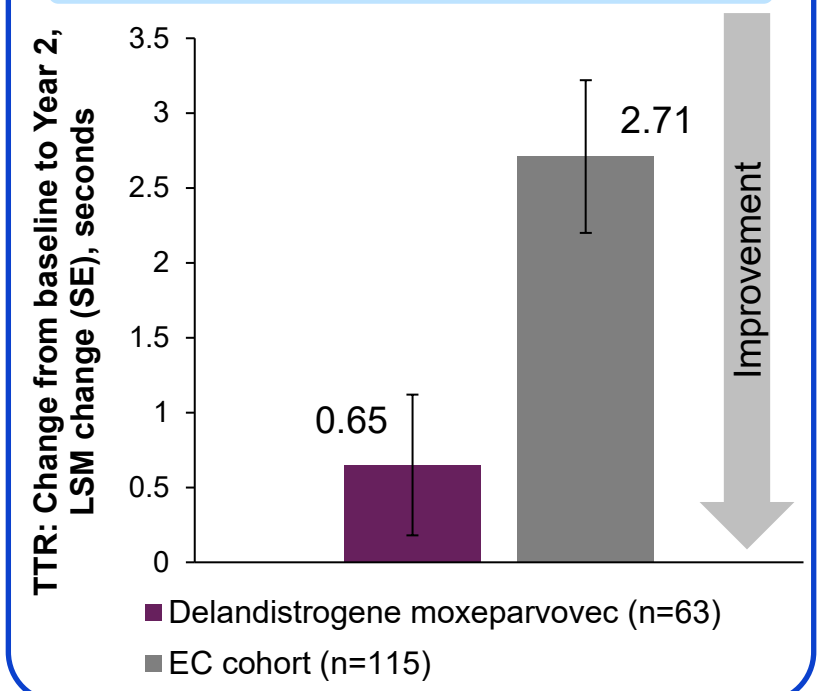
EMBARK Part 1: Functional outcomes at 2 years

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

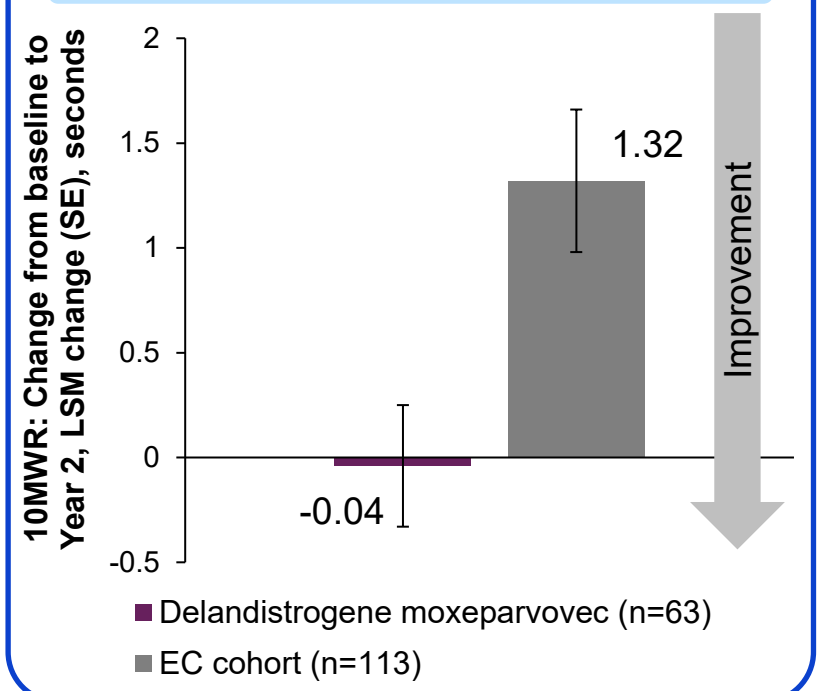
NSAA Δ 2.88 points; *P*=0.0001



TTR Δ –2.06 s; *P*=0.0033



10MWR Δ –1.36 s; *P*=0.0028



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.

EMBARK 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

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)

PDPF, percent dystrophin-positive fibers; SD, standard deviation.

EMBARK 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**

There were **no treatment-related AEs leading to death or discontinuation and there were no clinically significant complement-mediated AEs**

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)
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.

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

Evidence to date suggests **that most TR-TEAEs** were reported **within the first 90 days of infusion**

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

3-Year Functional Outcomes of Patients With Duchenne Muscular Dystrophy: Pooled Delandistrogene Moxeparvovec Clinical Trial Data Vs External Controls (P167)

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Presenter

Pooled analyses: Study methodology and analysis

Study 101 (NCT03375164)	Analysis	N=4
Study 102 (NCT03769116)	Analysis	n=26
ENDEAVOR Cohort 1 (NCT04626674)	Analysis	n=20

Delandistrogene moxeparvovec entry criteria:

- Received target dose of 1.33×10^{14} vg/kg
- ≥4 to <9 years old at baseline
- Had both baseline and Year 3 assessment values

Total: N=50

3-year pooled functional data of delandistrogene moxeparvovec were compared with an EC cohort of patients with DMD using propensity-score weighting* using a median regression model[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:

- ✓ ≥4 and <9 years at baseline
- ✓ NSAA total score ≥13 and ≤30 points
- ✓ TTR ≤10.4 seconds
- ✓ 10MWR time ≤9.1 seconds
- ✓ Stable dose of oral corticosteroid duration ≥12 weeks
- ✓ Had both baseline and Year 3 assessment values

Propensity-score weighting* 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 March 2025); 3. ClinicalTrials.gov. NCT01753804 (Accessed March 2025); 4. ClinicalTrials.gov. NCT00468832 (Accessed March 2025); 5. Spurney C, et al. *Muscle Nerve*. 2014; 50:250–256.

Pooled analyses: Demographics and baseline clinical characteristics

Baseline characteristics were well matched between the pooled delandistrogene moxeparvovec-treated patients and EC patients after propensity-score weighting*

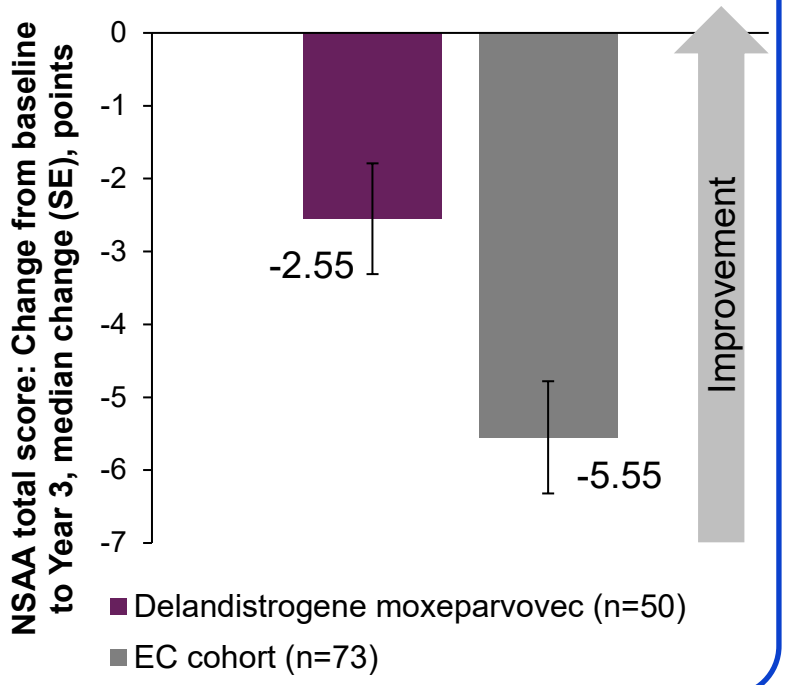
Characteristic, Mean (min, max)	Pooled delandistrogene moxeparvovec (N=50)†	EC cohort (N=73)†	Standardized mean difference after propensity-score weighting*
Age, years	6.37 (4.02, 8.89)	6.54 (4.75, 8.90)	–0.14
NSAA total score, points	22.3 (13, 30)	21.7 (13, 30)	0.15
TTR, time in seconds	4.35 (2.40, 10.40)	4.44 (2.00, 10.20)	–0.05
10MWR, time in seconds	5.10 (3.50, 9.10)	5.14 (3.60, 7.90)	–0.04
Weight, kg	22.74 (13.70, 34.50)	23.34 (15.90, 35.90)	–0.13
Height, cm	111.79 (94.40, 124.00)	112.87 (99.00, 130.20)	–0.14
BMI, kg/m^2	18.06 (13.16, 24.60)	18.18 (13.74, 22.52)	–0.06

*Inverse probability of treatment weighting. †N: Sample size (NSAA) after propensity-score weighting; prior to propensity-score weighting, there were 83 patients in the EC cohort who met the entry criteria and had at least 1 post-baseline visit (FOR-DMD, n=68; BioMarin PRO-DMD-01, n=12; CINRG DNHS, n=3).
10MWR, 10-meter Walk/Run; BMI, body mass index; EC, external control; NSAA, North Star Ambulatory Assessment; TTR, Time to Rise.

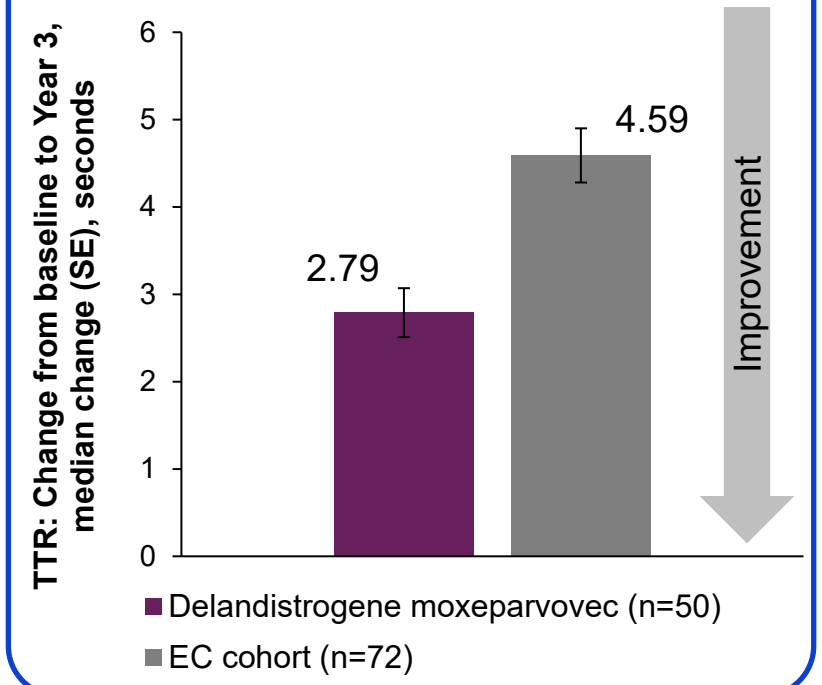
Pooled analyses: Functional outcomes at 3 years

At 3 years, **patients treated with delandistrogene moxeparvovec demonstrated functional benefit** versus the EC cohort

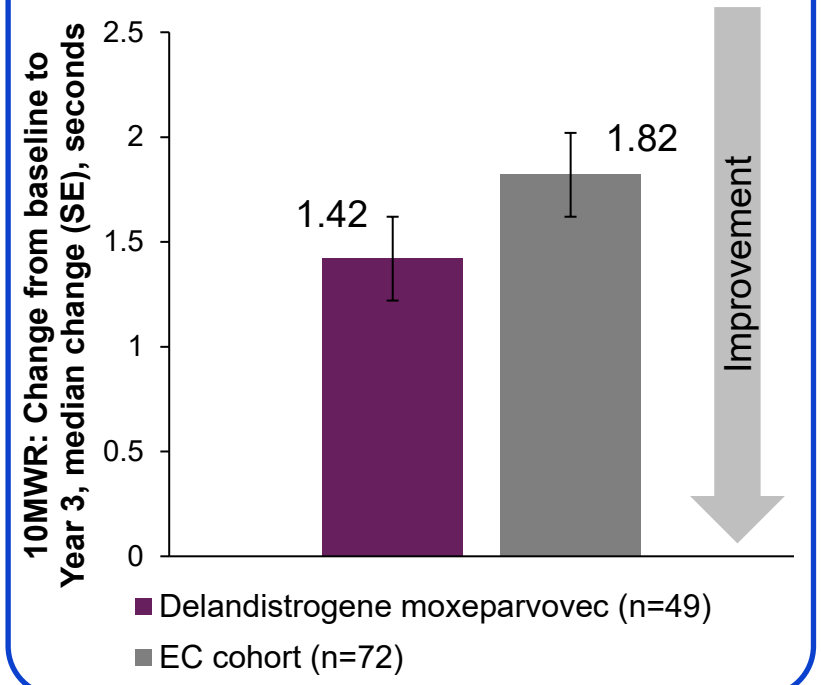
NSAA Δ 3.00 points; *P*=0.0003



TTR Δ –1.80 s; *P*<0.0001



10MWR Δ –0.40 s; *P*=0.0592



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

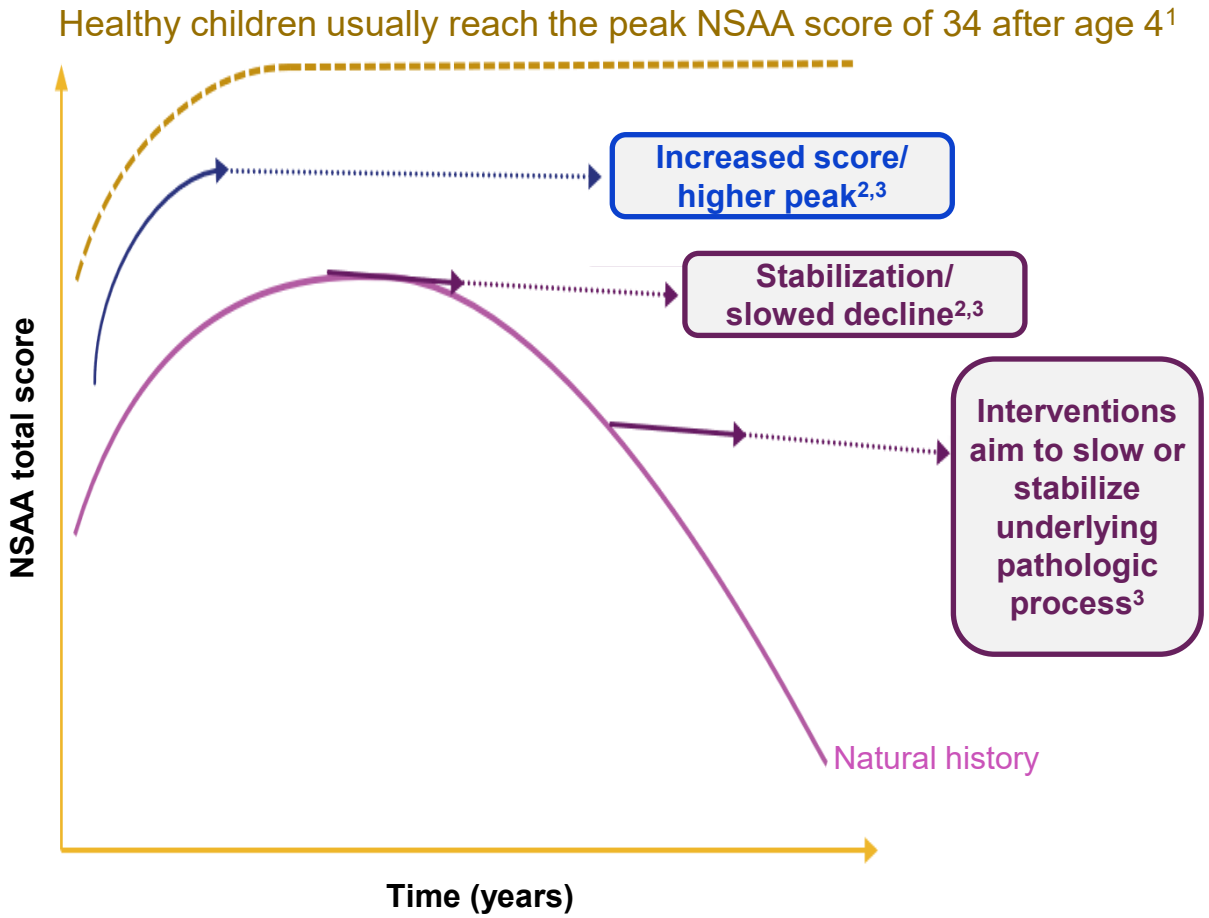
Conclusions

Results from 2-year EMBARK and 3-year pooled analyses indicate stabilization or slowing of disease progression compared with well matched ECs 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

>600 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.

QR codes

- QR code for EMBARK 2-Year poster (P169)

- QR code for Pooled Analyses 3-Year poster (P167)

EMBARK (P169): 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 Emily Turner, BSc, 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 while at Nationwide Children's Hospital at the time of the EMBARK study and is currently an employee of Sarepta Therapeutics. JRM is a co-inventor of AAVrh74.MHCK7.micro-dys technology
- FM has received honoraria and grants from Sarepta Therapeutics for participating at symposia and advisory boards and is involved as an investigator in Sarepta Therapeutics 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; and has a consultancy/advisory role with Biomarin, Capricor Therapeutics, Catalyst, Edgewise, Italfarmaco, PTC Therapeutics, F. Hoffmann-La Roche Ltd, Santhera Pharmaceuticals, and Sarepta Therapeutics. He has received honoraria from PTC Therapeutics and Sarepta Therapeutics
- EMM has received fees from AveXis, Biogen, and F. Hoffmann-La Roche Ltd
- EC has received honoraria from Sarepta Therapeutics for participating in advisory boards and grants as an investigator in Sarepta Therapeutics clinical trials
- HK has received grants from Sarepta Therapeutics, Pfizer, PTC Therapeutics, Taiho Pharmaceutical Co. Ltd, Chugai Pharmaceutical Co., Nippon Shinyaku Co. Ltd, and Kaneka Corporation. HK has received fees from Sarepta Therapeutics, Pfizer, PTC Therapeutics, Chugai Pharmaceutical Co., Nippon Shinyaku Co., and Kaneka Corporation
- CL-A is an investigator in Sarepta Therapeutics 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, 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, and Scholar Rock
- US-S has received honoraria for counseling and participating in invited talks from Sarepta Therapeutics 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, 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 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 and may have stock options. In addition, she is a co-inventor of AAVrh74.MHCK7.micro-dys technology

Pooled analyses (P167): Acknowledgements and disclosures

Acknowledgments

- The authors would like to thank the patients and their families for their participation in Study 101, Study 102 and ENDEAVOR, as well as the investigators and trial staff involved
- Study 101, Study 102 and ENDEAVOR are sponsored and funded by Sarepta Therapeutics, Inc., Cambridge, MA, USA. ENDEAVOR is also funded by F. Hoffmann-La Roche Ltd, Basel, Switzerland.
- Medical writing and editorial support were provided by Emily Turner, BSc, 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 has received study funding from Sarepta Therapeutics and has a service agreement with Sarepta Therapeutics to provide training on ongoing studies. JRM is a co-inventor of AAVrh74.MHCK7.micro-dys technology
- AMC has a consultancy/advisory role with Biohaven, Edgewise Therapeutics, Sarepta Therapeutics, and Scholar Rock and has received research funding from Biohaven, Edgewise Therapeutics, FibroGen, MDA, Sarepta Therapeutics, Inc., and Scholar Rock
- JD reports grants from AMO, Audentes, Avidity, Biogen, Cytokinetics, Ionis Pharmaceuticals, Novartis Gene Therapies, Roche Pharmaceuticals, Sanofi-Genzyme, Sarepta Therapeutics, and Scholar Rock. JWD participates on advisory boards and is a consultant for Affinia Therapeutics, AMO Pharmaceuticals, Astellas Gene Therapies, Audentes Therapeutics, Avidity Therapeutics, Biogen, Cytokinetics, Epirium Bio, Ionis Pharmaceuticals, Kate Therapeutics, Novartis, Novartis Gene Therapies, Pfizer, Roche/Genentech Pharmaceuticals, Sarepta Therapeutics, Scholar Rock, Shift Therapeutics, and Vertex. JWD participated in the PepGen Scientific Advisory Board (2021). JWD was a paid advisor to the Muscular Dystrophy Association and an unpaid advisor to Myotonic Dystrophy Foundation, CureSMA, SMA Foundation, Parents Project Muscular Dystrophy, Foundation Building Strength for Nemaline Myopathy, Cure CMD, and Solve FSHD. JWD holds patents licensed to Athena Diagnostics for genetic testing of myotonic dystrophy type 2 (US patent 7442782) and spinocerebellar ataxia type 5 (US patent 7527931)
- CMM reports grants from Capricor Therapeutics, Catabasis, Edgewise Therapeutics, Epirium Bio, Italfarmaco, Pfizer, PTC Therapeutics, Santhera Pharmaceuticals, Sarepta Therapeutics; and others from Capricor Therapeutics, Catabasis, PTC Therapeutics, Santhera Pharmaceuticals, and Sarepta Therapeutics
- CP participates on an advisory board and is a consultant for Biogen, Sarepta Therapeutics, AveXis/Novartis Gene Therapies, Genentech/Roche, and Scholar Rock; serves as a speaker for Biogen; is PI of studies sponsored by AveXis/Novartis Gene Therapies, AMO, Astellas, Biogen, CSL Behring, FibroGen, PTC Therapeutics, Pfizer, Sarepta Therapeutics, and Scholar Rock
- PS reports being a consultant/independent contractor for AveXis, Biogen, Cytokinetics, and Sarepta Therapeutics, and receiving grants/research support from AveXis, Biogen, Cytokinetics, Ionis Pharmaceuticals, Sanofi Genzyme, and Sarepta Therapeutics
- CMZ receives research support from Biogen and Novartis, serves on an advisory board for Biogen, receives speaker fees from Sarepta Therapeutics, and was a paid consultant for Optum
- MF, KD, and JSE are employees of Sarepta Therapeutics and may have stock options
- CR and APM are employees of Roche Products Ltd and may have stock options in F. Hoffmann-La Roche Ltd
- LRR-K is an employee of Sarepta Therapeutics and may have stock options. In addition, she is a co-inventor of AAVrh74.MHCK7.micro-dys technology