SAREPTA THERAPEUTICS

SRP-9001-301 PART-1 3-Year Data Topline Results

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Forward-looking Statements

This presentation contains “forward-looking statements.” Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “believe,” “anticipate,” “plan,” “expect,” “will,” “may,” “intend,” “prepare,” “look,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements relating to our future operations; our technologies, strategies, and priorities; ELEVIDYS; and our clinical trials, including Study 9001-301.

Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: different methodologies, assumptions and applications we use to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe the data collected from clinical trials are positive, the results of future research may not be consistent with past positive results, or may fail to meet regulatory approval requirements for the safety and efficacy of our products; we may observe adverse reactions in our clinical trials or in patients who receive our approved products; our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our business; our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; our products or product candidates may cause undesirable side effects that result in significant negative consequences following any marketing approval; we may not be able to comply with all FDA requests in a timely manner or at all; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; and those risks identified under the heading “Risk Factors” in our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company, which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company’s business, results of operations and the trading price of Sarepta’s common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained herein. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except as required by law.

EMBARK (Study 9001-301)

Louise Rodino-Klapac, PhD

President, R&D and Technical Operations

3-year Topline Functional Results from Part 1 of EMBARK (Study 9001-301)

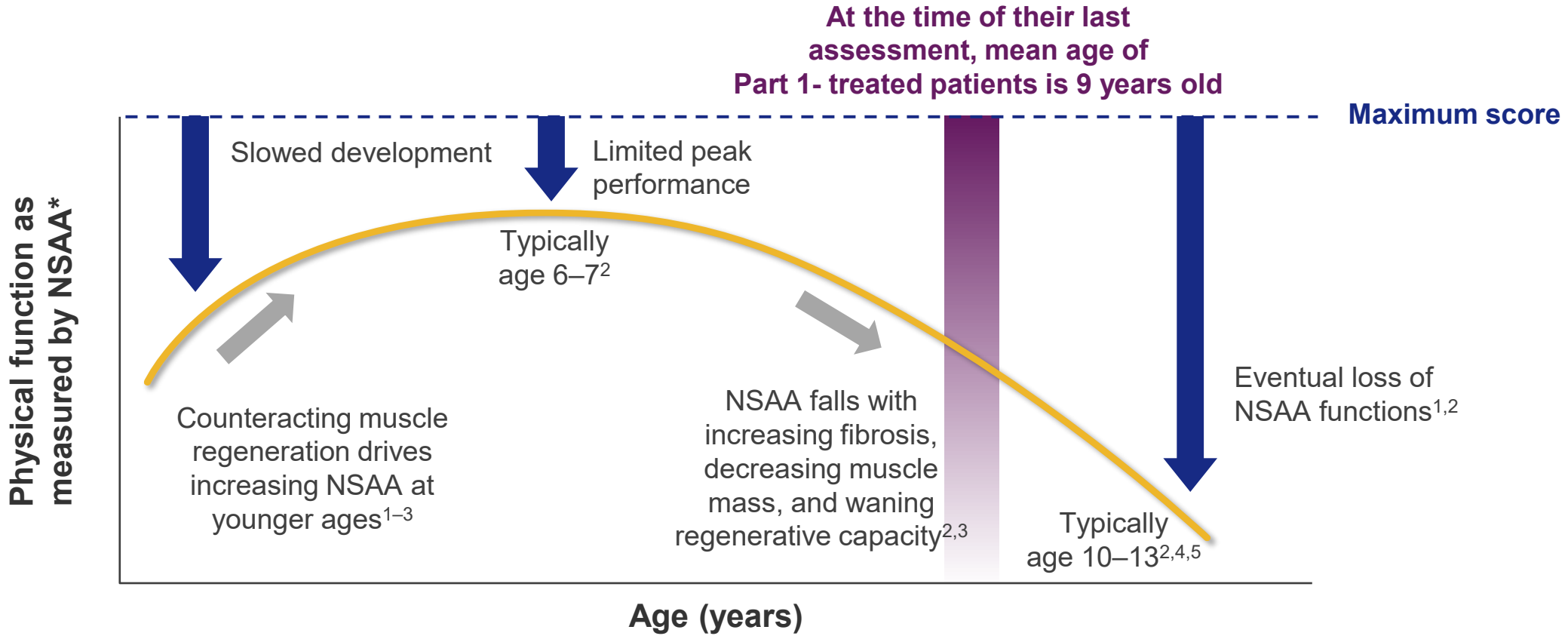
- On average, ELEVIDYS-treated patients remain above their baseline three years after treatment as measured by NSAA

- Statistically significant 73% slowing of disease progression as measured by TTR (time to rise)

- Statistically significant 70% slowing of disease progression as measured by 10MWR (10-meter walk run)

For the first time, a gene therapy for Duchenne has demonstrated a profound change in the trajectory of the disease out to three years

Physical Function Typically Peaks Around Age 6 in Individuals With Duchenne[1-3]



*NSAA is a composite endpoint evaluating physical function in ambulant children with Duchenne.[10]

NSAA, North Star Ambulatory Assessment.

1. McDonald CM, *et al. Lancet*. 2018;391(10119):451–61. 2. Muntoni F, *et al. PLoS One*. 2019;14(9):e0221097. 3. Rooney WD, *et al. Neurology*. 2020;94(15):e1622–33. 4. Emery AEH. *Lancet*. 2002;359(9307):687–95. 5. Niks EH and Aartsma-Rus A. *Expert Opin Biol Ther*. 2017;17(2):225–36. 6. Goemans N, *et al. Neuromuscul Disord*. 2013;23(8):618–23.

Muscle Biopsies of Patients with DMD Show Continued Damage at Early Ages

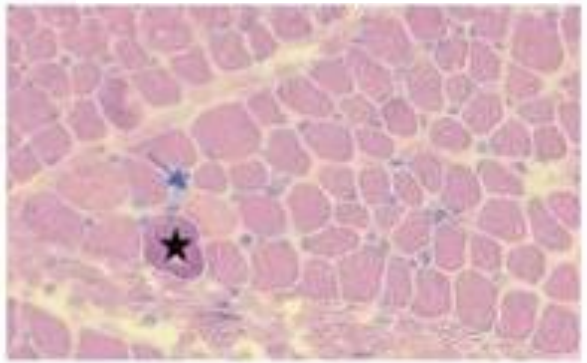
Biopsies Showed Fiber Size Variation, and Progressive Loss of Muscle Structure and Integrity[a,b]

Blue arrows represent necrotic fibers

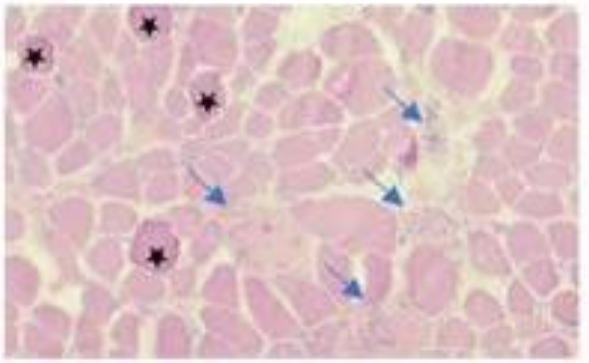
Black stars represent hypercontracted fibers

Control Quadricep 4 Years

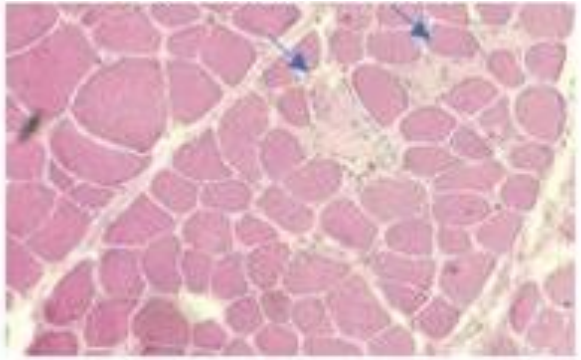
DMD Quadricep 1 Year



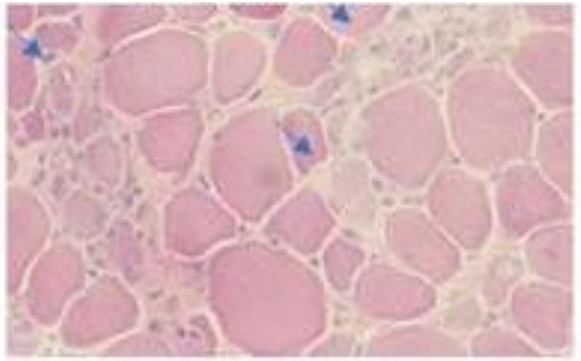
DMD Quadricep 3 Years



DMD Quadricep 5 Years

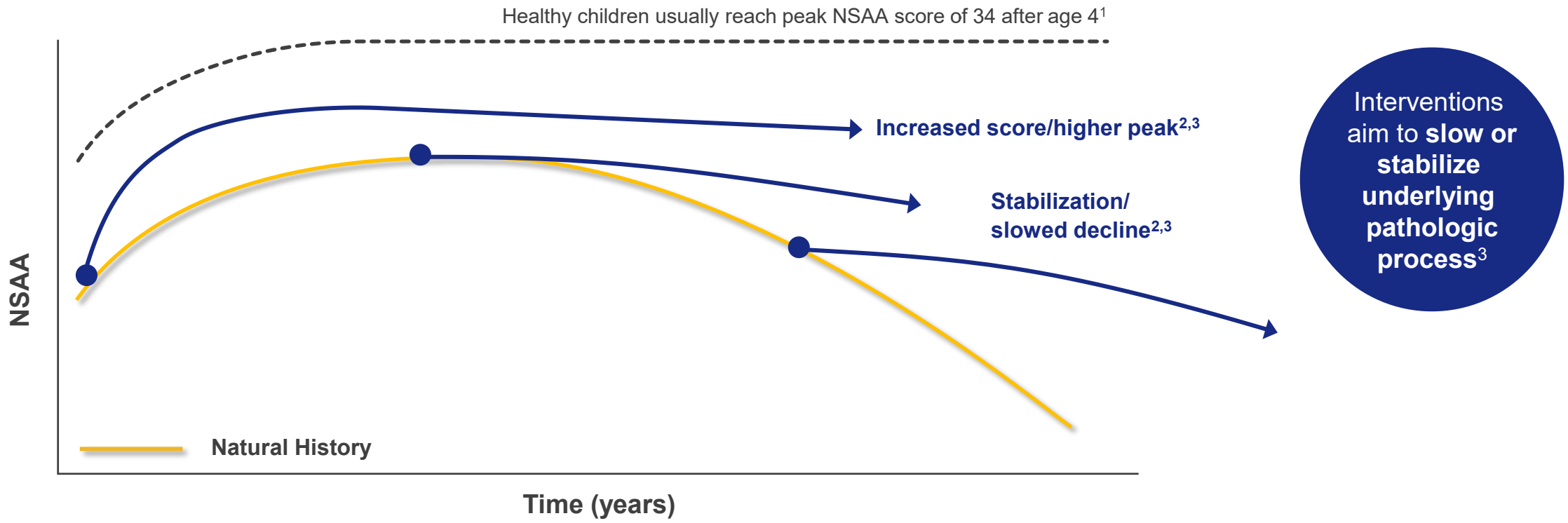


DMD Quadricep 8 Years



[a]Hematoxylin & Eosin (H&E) staining [b]Biopsy images shown are each from different patients
DMD, Duchenne muscular dystrophy. Cardone N, et al. *Acta Neuropathol Commun.* 2023;11(1);167.

Potential Outcomes Based on Timing of Therapeutic Intervention When Attempting to Stabilize Duchenne Progression



Change from baseline without context of expected course of disease may not be an appropriate method to assess treatment outcomes.[2]

1. Mercuri E, et al. *PLoS One*. 2016;11(8):e0160195. 2. Muntoni F, et al. *PLoS One*. 2019;14(9):e0221097. 3. McDonald CM, et al. *Lancet*. 2018;391(10119):451-461.

Largest and Most Comprehensive Clinical Program in Duchenne

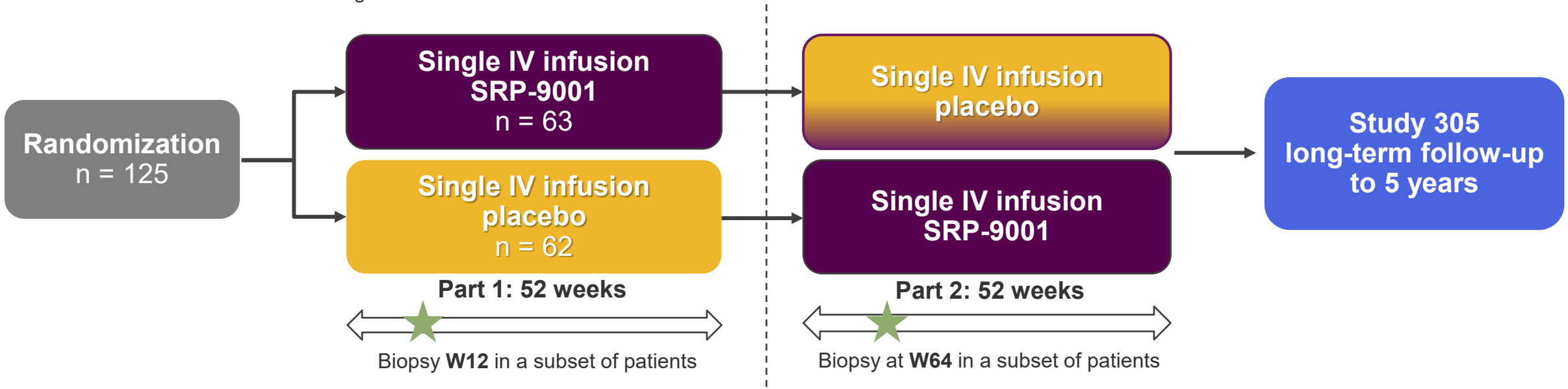
Clinical trial participants have ranged in age from 2 to 24 years old, with body weights ranging from 12 kg to close to 90 kg

SRP-9001-301 Study Design

EMBARK

A completed Phase 3, multinational, double-blind, randomized, placebo-controlled study evaluating the safety and efficacy of ELEVIDYS (delandistrogene moxeparvovec-rokl), in boys with DMD aged 4–7 years old

Key functional inclusion criteria:
- NSAA score 17-28 (inclusive) at Screening
- Time to Rise < 5 sec at Screening



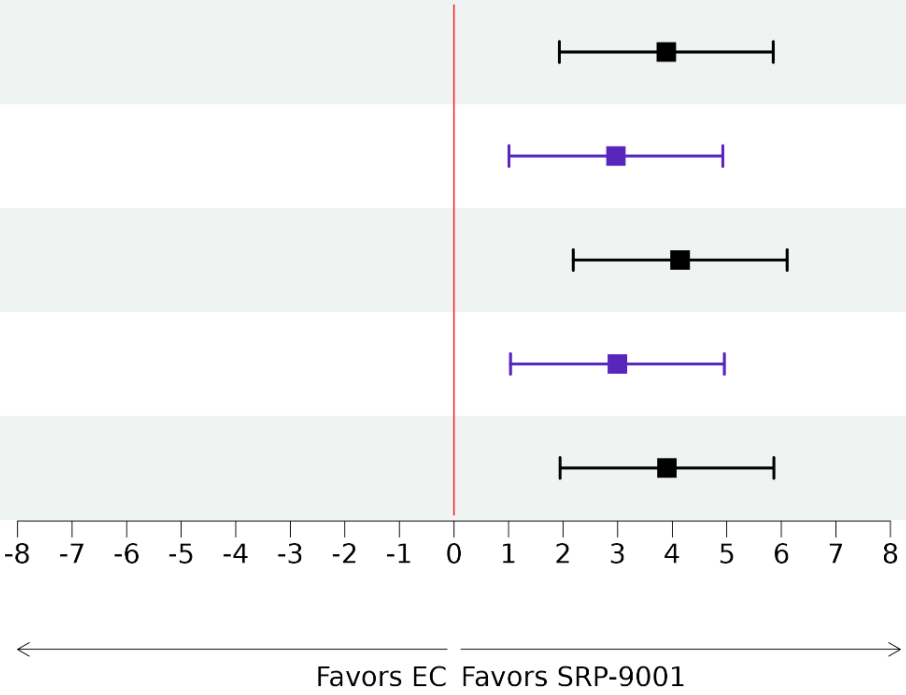
Primary endpoint:
- Change in North Star Ambulatory Assessment (NSAA) total score from Baseline to Week 52

Key Secondary endpoints:
- Quantity of SRP-9001 dystrophin protein expression, as measured by WB, at Week 12
- Change in time to rise (TTR) from floor from Baseline to Week 52
- Change in 10-meter timed test (10MTT) from Baseline to Week 52

Other timed secondary endpoints:
- Stride Velocity 95th Centile (SV95C)
- 100-meter walk/run
- Ascend 4 Steps

EMBARK Part 2: Statistically Significant, Clinically Meaningful Differences 2 years After Treatment Compared to External Control

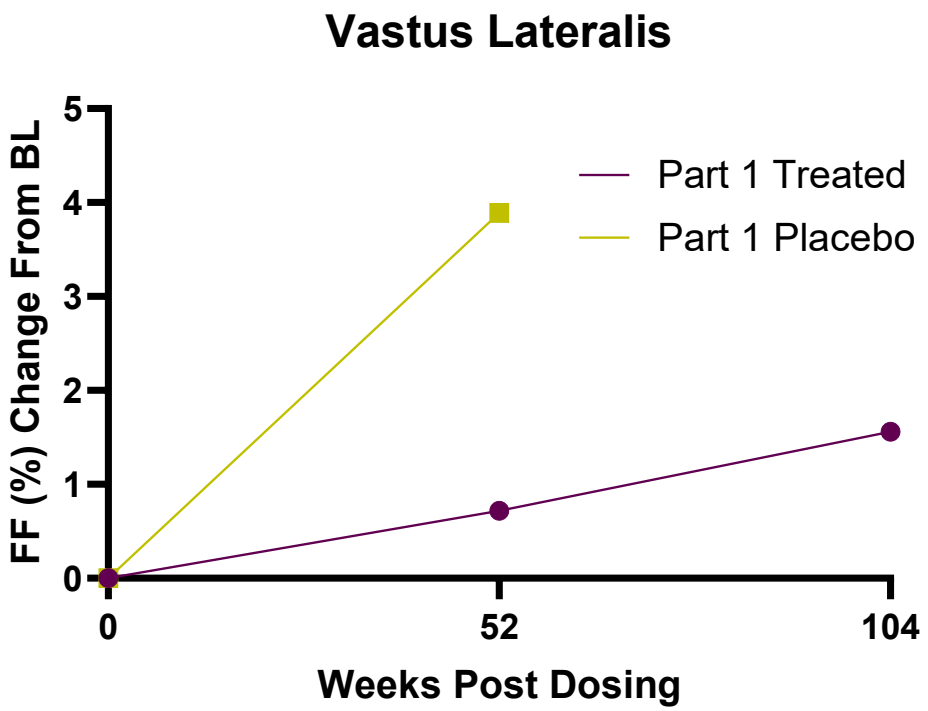
Endpoint	SRP-9001	EC	LSMean Diff (95% CI)	P-value
NSAA	63	114	2.88 (1.43, 4.33)	0.0001
TTR*	63	115	-2.06 (-3.43, -0.7)	0.0033
TTR velocity	63	115	0.055 (0.03, 0.081)	<.0001
10MWR time*	63	113	-1.36 (-2.24, -0.47)	0.0028
10MWR velocity	63	113	0.239 (0.118, 0.359)	0.0001

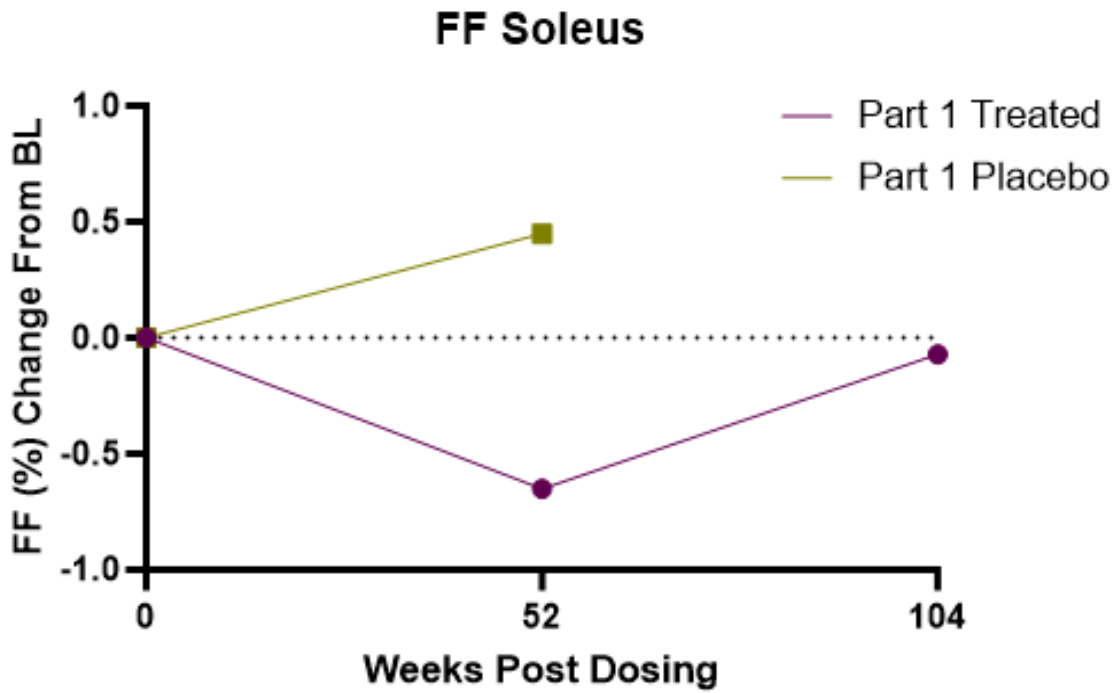


** Sign reversed to align favorable directions among effect endpoints*

Diff in Means (of change from baseline) and confidence intervals are standardized by dividing by the standard error (SE). The half width of the forest plot bars will be 1.96. Numerical results of the Diff in Means are on original scale (without SE adjustment) along with P-values (unadjusted nominal). RFFT and TMWRT signs are reversed in forest plot to align favorable directions among endpoints. Numerical results of Diff in Mean kept original sign.

2 Year Results: Musculoskeletal MRI - Fat Fraction





Placebo	N=16	N=14	
Treated	N=15	N=14	N=14

N=17	N=15	
N=16	N=16	N=15

Stabilization in MRI muscle FF in ELEVIDYS treatment group with two years values below those seen in part 1 placebo group

1. Naarding KJ, Reyngoudt H, van Zwet EW, etal. MRI vastus lateralis fat fraction predicts loss of ambulation in Duchenne muscular dystrophy. Neurology. 2020 Mar 31;94(13):e1386-e1394.
2. Barnard AM, Willcocks RJ, Triplett WT, etal. MR biomarkers predict clinical function in Duchenne muscular dystrophy. Neurology. 2020 Mar 3;94(9):e897-e909.
3. Willcocks RJ, Rooney WD, Triplett WT, etal. Multicenter prospective longitudinal study of magnetic resonance biomarkers in a large duchenne muscular dystrophy cohort. Ann Neurol. 2016 Apr;79(4):535-47. doi: 10.1002/ana.24599. Epub 2016 Feb 19. PMID: 26891991; PMCID: PMC4955760.

Cardiac MRI Shows Patients Remain in Normal Range Over 2 Years of Follow-up

No statistical or clinical differences in these groups

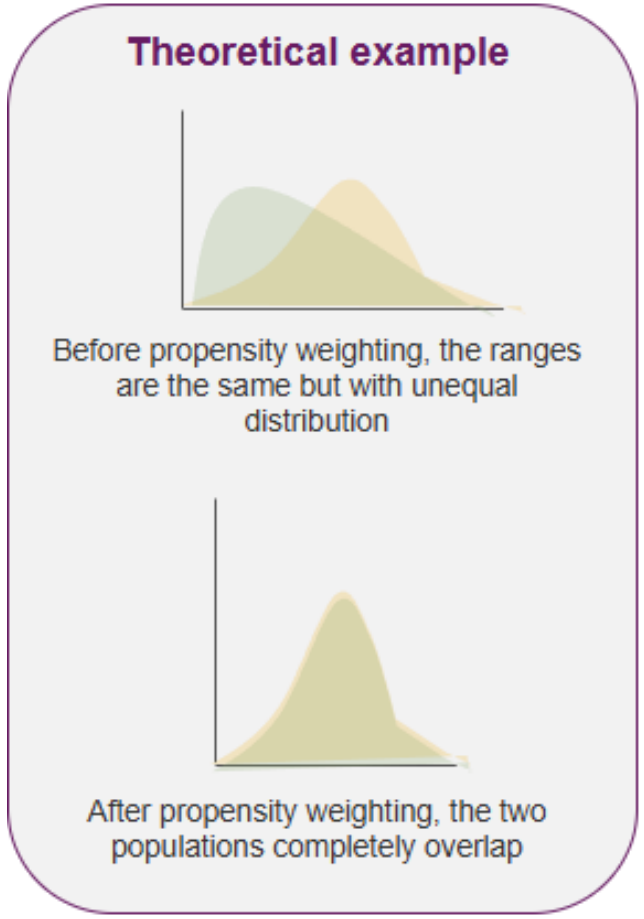
	Part 1 Treated: Baseline	Year 1 post-treatment	Year 2 post-treatment	Crossover Treated: Baseline	Year 1 post-placebo	Year 1 post-treatment
Subjects (N)	16	16	14	19	16	18
Left Ventricular Ejection Fraction, mean (range)	64.69% (54-72)	65.25% (55-74)	61.73% (52-72)	64.58% (47-74)	66.38% (52-74)	63.37% (58-75)
Global Circumferential Strain, mean (range)	-18.37% (-22.15 to -12.96)	-18.82% (-21.17 to -15.6)	-18.21% (-20.62 to -12.90)	-18.59% (-23.53 to -13.00)	-19.11% (-23.97 to -12.94)	-19.26% (-23 to -11.90)

Results from EMBARK:
3-Year Topline Data, Part 1-treated vs EC
(Analysis Using LS-Mean by MMRM)

James Richardson, MA (Oxon), BMBCh, MBA, MRCP (Lon)
Executive Vice President and Chief Medical Officer

Background on the External Control (EC)

- In the absence of a placebo arm after Part 1, the EMBARK study protocol and statistical analysis plan (SAP) are used to create a **pre-specified**, propensity-weighted external control to contextualize study level results
- External control cohorts chosen through pre-specified selection criteria and pre-specified data sources:
  - Eli Lilly Tadalafil placebo (n= 116)
  - DEMAND-III placebo arm (n= 186)
  - PRO-DMD (n= 269)
  - CINRG DNHS (n= 440)
  - FOR-DMD (n= 194)
- Propensity score weighting is used to decrease bias
- Protocols agreed to by regulatory authorities prior to the start of the study



Baseline Characteristics Balanced for Part 1-treated and EC

Baseline Characteristics (Weighted) Full Analysis Set

Baseline	ELEVIDYS Mean (Min, Max)	External Control Mean (Min, Max)
Age	5.98 (4.07, 7.87)	6.24 (4.24, 7.99)
NSAA	23.3 (14, 32)	23.5 (15, 32)
Time to Rise	3.51 (1.85, 5.75)	3.52 (1.90, 5.70)
10MWR Time	4.80 (3.20, 6.85)	4.78 (3.00, 6.70)
Weight	21.20 (13.5, 37.4)	21.18 (14.0, 36.0)
Height	108.65 (93.5, 127.0)	110.60 (94.9, 131.1)
BMI	17.80 (13.69, 24.92)	17.90 (13.74, 23.64)

Balanced baseline covariates after propensity score weighting

Disposition

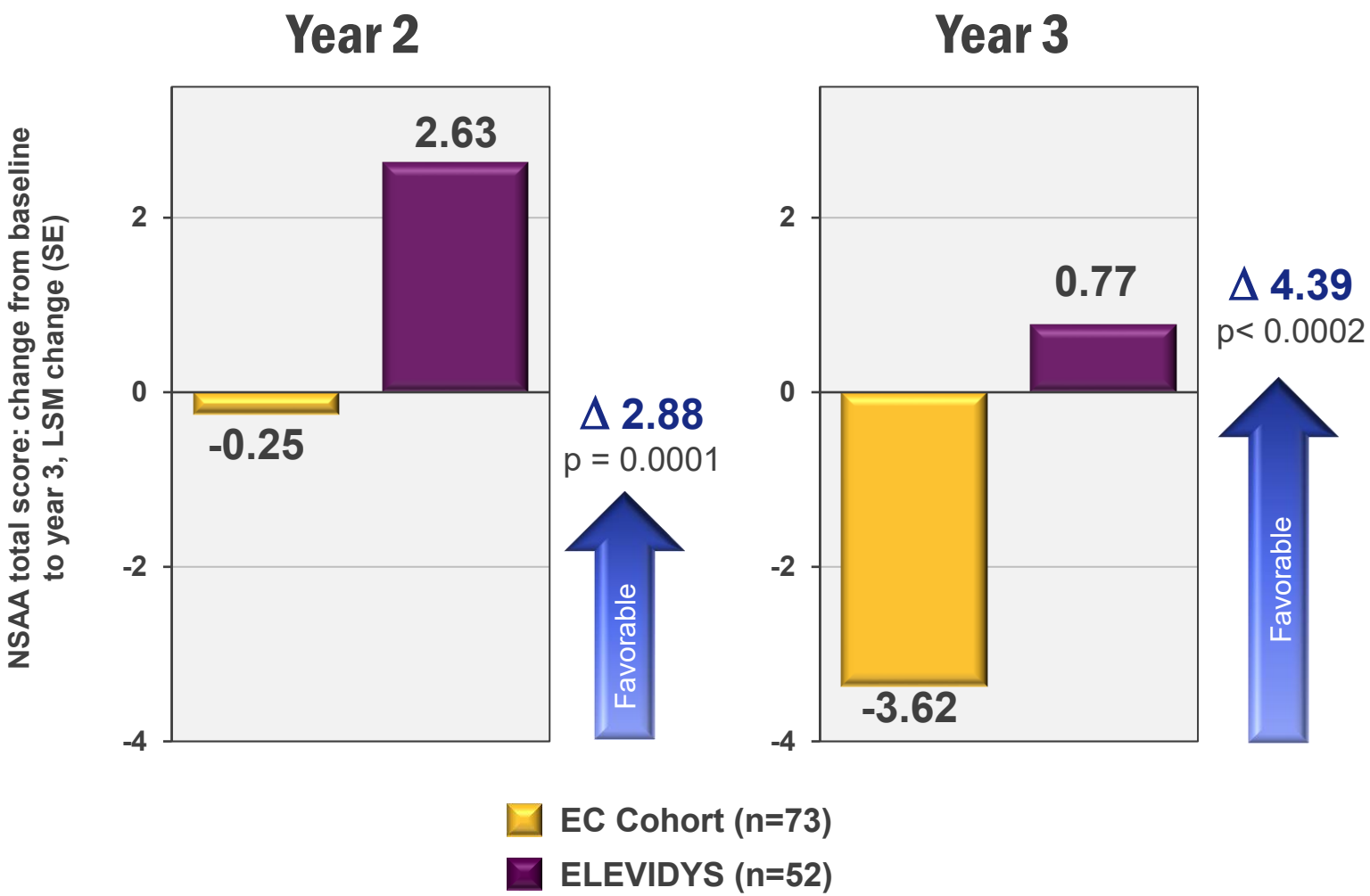
	Cohort	Baseline	Year 1	Year 2	Year 3
		N*	N*	N*	N*
Overall	External Control	143	141	114	73
	ELEVIDYS	**64**	**64**	**63**	**52**

70+ EC patients at 3 years reinforces the rigor of this analysis

***N**: sample size (NSAA) after PS weighting

ELEVIDYS-treated patients (n=52) achieved a significantly higher NSAA total score compared to the external control group, with a p-value of 0.0002

Mean age of patients 9 years old at time of last assessment

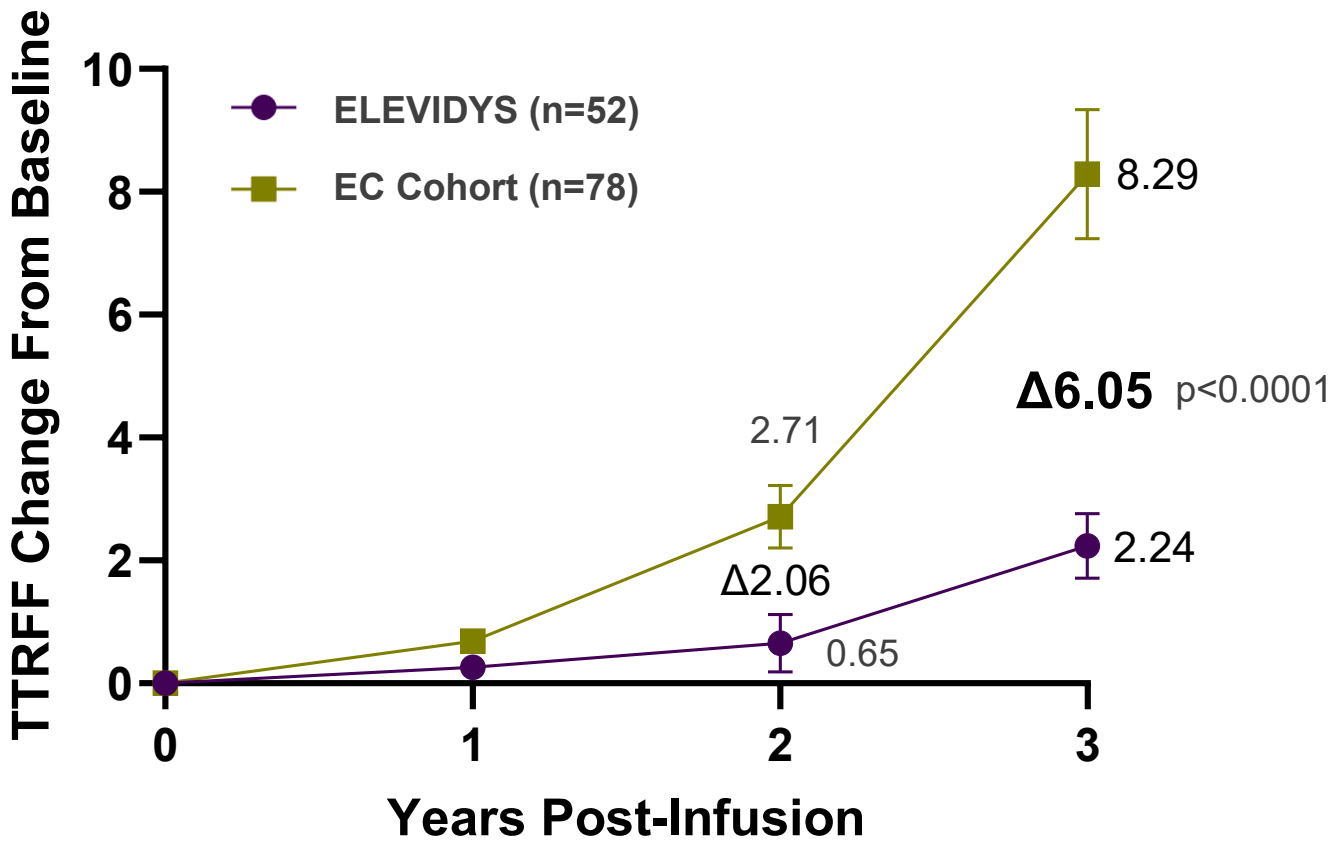


Key Observations:

- The functional gap between treated and EC groups grew from a 2.88-point difference at Year 2 to a 4.39-point difference at Year 3.
- On average, Elevidys-treated patients remain above their baseline while untreated peers declined to 3.62 points below baseline.

ELEVIDYS-treated patients demonstrated a highly significant 73% slowing of disease progression on TTR, with a p-value of < 0.0001

Mean age of patients 9 years old at time of last assessment

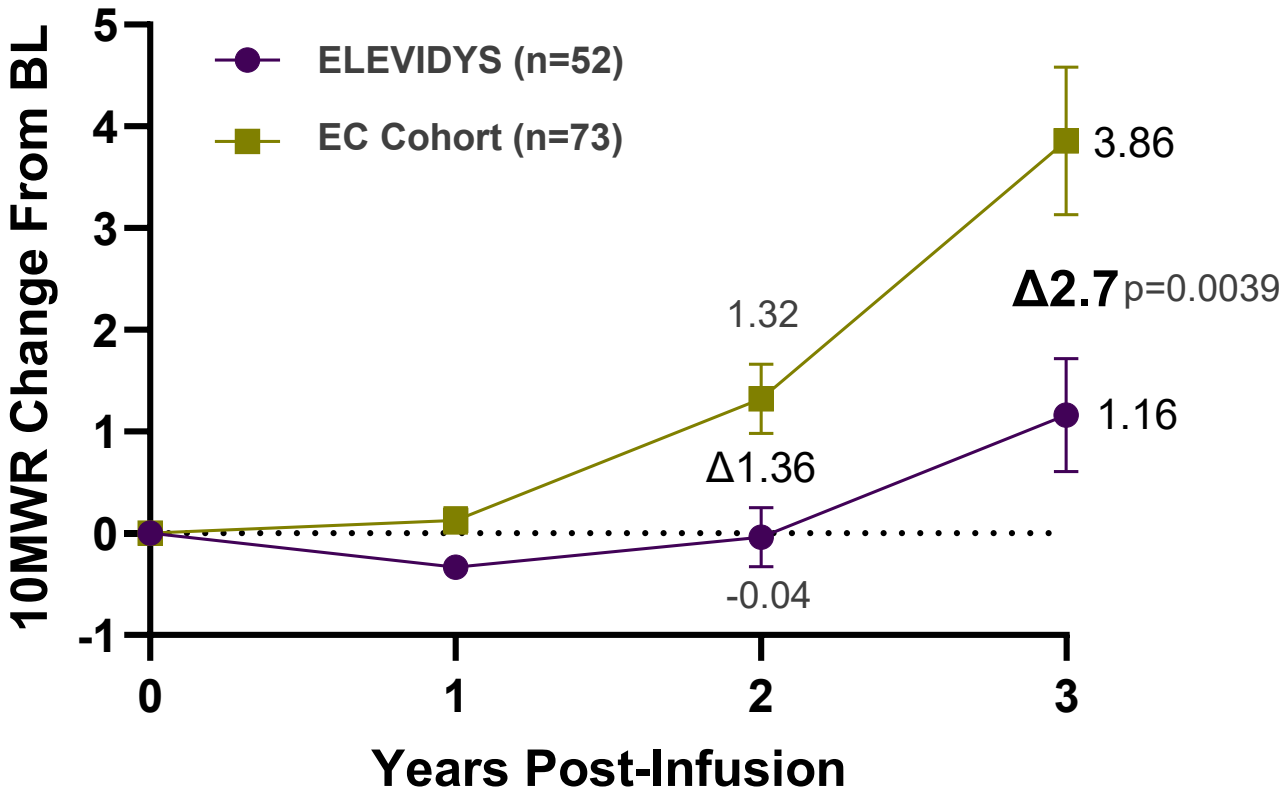


Key Observations:

- The performance gap nearly tripled in the third year, expanding from a 2.06-second difference at Year 2 to a 6.05-second difference at Year 3.
- ELEVIDYS-treated patients were able to far more rapidly rise from floor as compared to their untreated peers, who took an average of 8.29 seconds longer than baseline to stand compared to just 2.24 seconds for the treated group.

ELEVIDYS-treated patients achieved a statistically significant 70% slowing of disease progression on 10MWR, with a p-value of 0.0039

Mean age of patients 9 years old at time of last assessment



Key Observations:

- Treated patients remained stable, with a decline of 1.16 seconds from baseline, whereas the untreated group declined over 3 times as much declining 3.86 seconds from their original time.

Three-year Safety for Part 1 Treated patients

	Year 1 (EMBARK Y1)	Year 2 (EMBARK Y2)*	Year 3 (EXPEDITION Y1)^
Patients with any SAEs	14	5	4
Patients with treatment-related SAEs	7	1	0
Treatment-related deaths	0	0	0Ŧ

- No new safety signals were observed
- The safety profile of ELEVIDYS as observed in participants dosed in EMBARK is fully consistent with our understanding of the identified and labelled risks of the medicine

*Only includes new events in year 2 of the study, excludes unresolved events that began in year 1
^Only includes new events in year 3 of follow-up, excludes unresolved events that began prior to this period
ŦOne treatment unrelated death of a Part 1-treated patient was disclosed in the community safety update webinar in August 2025 and on regulations.gov: https://www.regulations.gov/comment/FDA-2025-P-1929-0007

Long-term results from EMBARK demonstrate significant and growing functional benefit compared to EC

- At the time of their last assessment, mean age of patients treated in EMBARK is 9 years old, corresponding to a nearly universal period of functional decline in Duchenne
- On average, ELEVIDYS-treated patients remain above their baseline three years after treatment as measured by NSAA
- Statistically significant 73% slowing of disease progression as measured by TTR (time to rise)
- Statistically significant 70% slowing of disease progression as measured by 10MWR (10-meter walk run)
- Safety profile remains consistent

Dr. Crystal Proud

Chief of Neurology and Director of Neuromuscular Medicine

Children’s Hospital of The King’s Daughters

Q&A

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