Patients can’t wait for the next breakthrough in medical research.

So neither will we.

Doug Ingram
President and CEO

Louise Rodino-Klapac, PhD
Chief Scientific Officer and Head of R&D

EMBARK Topline Data
October 30, 2023

SAREPTA THERAPEUTICS

BENJAMIN
Living with Duchenne muscular dystrophy

Forward Looking Statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, forward-looking statements will be made during this conference call. Any statements made by Sarepta 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, business plans, priorities, research and development programs; the potential benefits of ELEVIDYS, including its potential to be a disease-modifying therapy; our understanding that FDA is committed to evaluating a labeling expansion to the fullest extent possible based on a review of the data and will do so rapidly; and expected timelines, plans and milestones, including submitting efficacy supplement as soon as possible seeking label expansion to treat all patients with Duchenne, submitting a PMR seeking transition from accelerated approval to traditional approval, and pursuing presentation and publication of full results from EMBARK in future scientific forums.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control. 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: the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations; the FDA may not approve a supplement to expand the approved label for ELEVIDYS; we may not be able to comply with all FDA requests in a timely manner or at all; our data may not be sufficient for obtaining regulatory approval; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and 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 product candidates; the commencement and completion of our clinical trials and announcement of results may be delayed or prevented for a number of reasons, including, among others, denial by the regulatory agencies of permission to proceed with our clinical trials, or placement of a clinical trial on hold, challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials and inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials; 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 of our product candidates are positive, these data may not be sufficient to support approval by the FDA or other global regulatory authorities; we may not be able to execute on our business plans, including meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, for various reasons, many of which may be outside of our control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; and those risks identified under the heading "Risk Factors" in our most recent Annual Report on Form 10-K for the year ended December 31, 2022 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. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

Welcome and Introduction

Doug Ingram

Topline Results

Louise Rodino-Klapac, PhD

Study Design

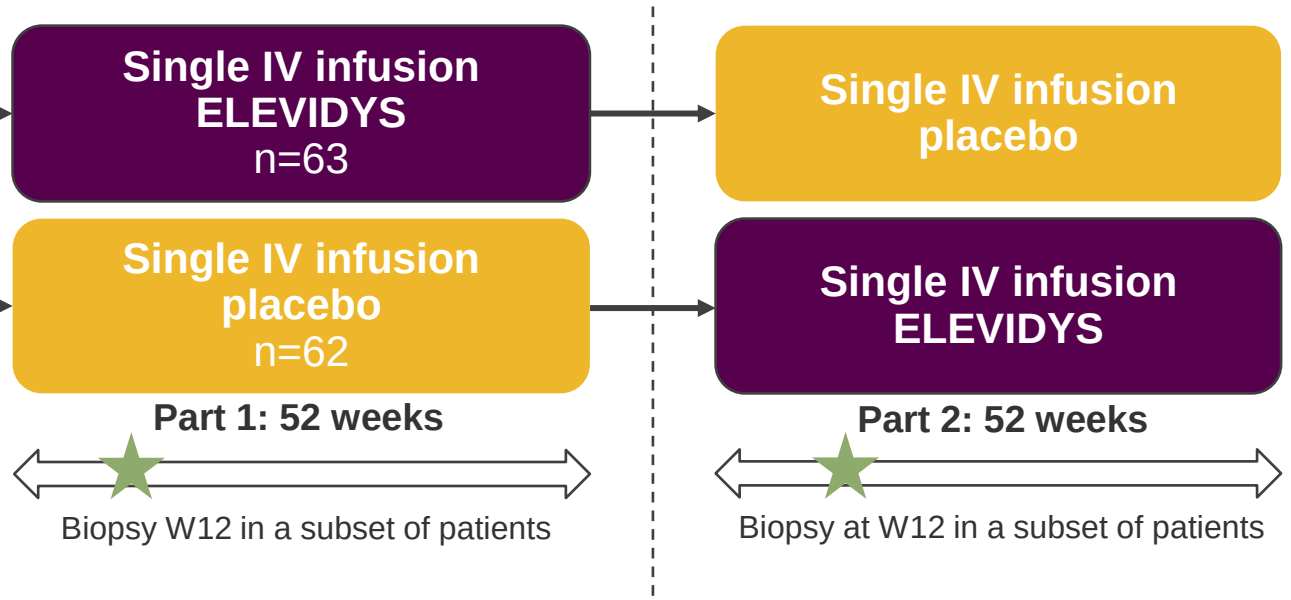
EMBARK (Study SRP-9001-301): Trial design

An ongoing Phase 3 multinational double-blind, randomized, placebo-controlled study evaluating the safety and efficacy of ELEVIDYS compared to placebo in boys with DMD aged 4-7 years old

Key functional inclusion criteria:
NSAA 17 to 28 inclusive
TTR <5 seconds

Randomization
n=125

Stratification based on:
Age (4-5yo vs 6-7yo)
NSAA at screening (≤22 vs >22)



Primary endpoint:
- Change in NSAA total score from Baseline to Week 52

Key secondary endpoints:
- Quantity of ELEVIDYS 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 walk/run (10MWR) from Baseline to Week 52

Other timed secondary endpoints:
- Stride velocity 95th centile (SV95C)
- 100-meter walk/run (100MWR)
- Ascend 4 steps

Treated and placebo group demographics were well-matched at baseline

Characteristic	Statistics	Placebo (n=62)	ELEVIDYS (n=63)
Age (years)	Mean (SD) Min, Max	6.08 (1.05) 4.03, 7.99	5.98 (1.06) 4.07, 7.87
Age, 4-5 years	n (%)	29 (46.8)	30 (47.6)
Race, White	n (%)	46 (74.2)	49 (77.8)
Ethnicity, Hispanic	n (%)	8 (12.9)	15 (23.8)
Height (cm)	Mean (SD) Min, Max	110.68 (7.44) 95.2, 127.5	108.64 (6.74) 93.5, 127.0
Dosing Weight (kg)	Mean (SD) Min, Max	22.37 (6.42) 14.4, 41.6	21.29 (4.62) 13.5, 38.5
BMI (kg/m^2)	Mean (SD) Min, Max	17.89 (3.20) 13.45, 26.86	17.85 (2.20) 13.69, 24.92
Years since diagnosis of DMD	Mean (SD) Min, Max	2.60 (1.78) 0.24, 7.55	2.62 (1.73) 0.00, 6.71
Years since corticosteroid treatment started	Mean (SD) Min, Max	0.97 (0.83) 0.24 4.01	1.07 (0.92) 0.23, 6.17
Genetic mutation **Large deletion** **Large duplication** **Small mutation**	n (%)	 41 (66.1) 3 (4.8) 18 (29.0)	 45 (71.4) 3 (4.8) 15 (23.8)

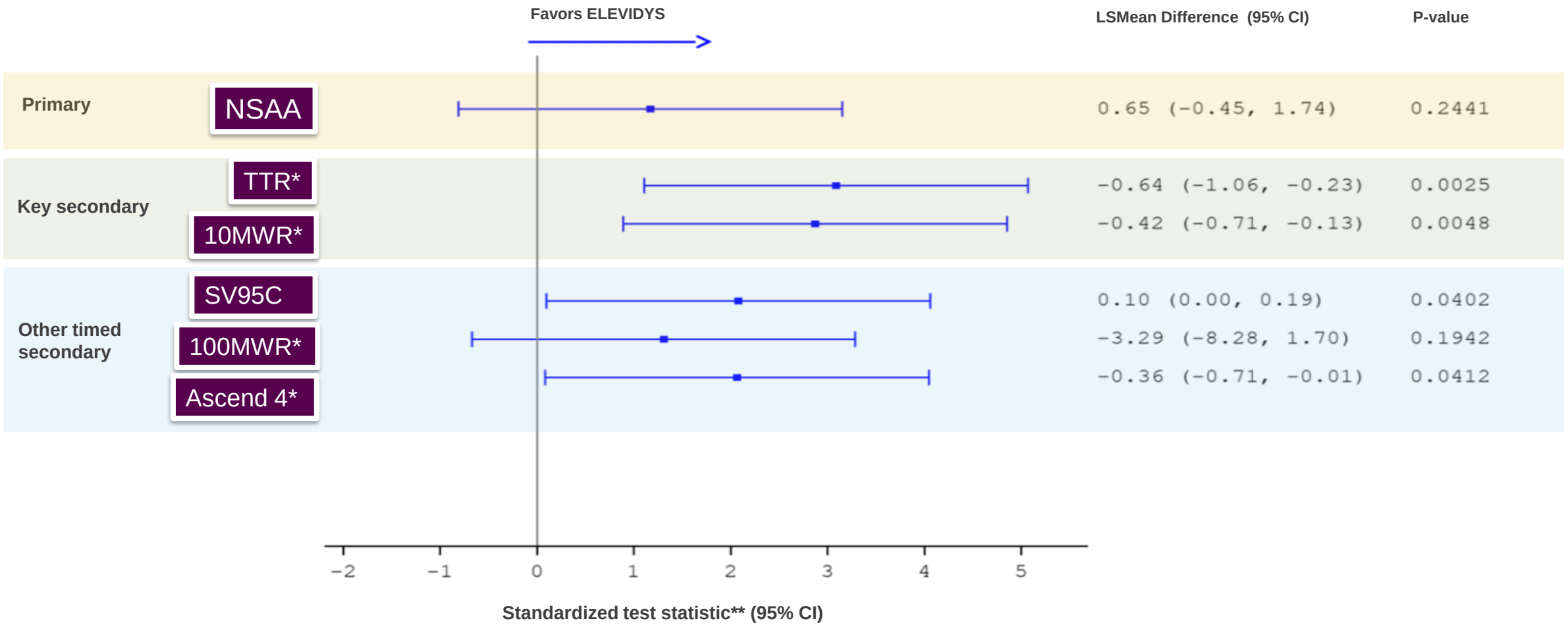
Treated and placebo group functional abilities well-matched at baseline

Characteristic	Statistics	Placebo (n=62)	ELEVIDYS (n=63)
NSAA total score	Mean (SD) Min, Max	22.82 (3.78) 15.5, 30.0	23.10 (3.75) 14, 32
Time to Rise (seconds)	Mean (SD) Min, Max	3.60 (0.68) 2.25, 5.00	3.52 (0.81) 1.85, 5.75
10-meter walk/run (seconds)	Mean (SD) Min, Max	4.92 (0.73) 3.65, 7.00	4.82 (0.79) 3.20, 6.85
Stride velocity 95th centile (m/sec)	Mean (SD) Min, Max	1.77 (0.29) 1.10, 2.35	1.82 (0.30) 1.06, 2.50
100 meter (seconds)	Mean (SD) Min, Max	63.01 (17.01) 38.7, 118.1	60.67 (15.55) 38.0, 129.2
Ascend 4 steps (seconds)	Mean (SD) Min, Max	3.37 (1.09) 1.5, 7.1	3.17 (1.01) 1.6, 7.1

Topline Results

Results favor treatment with ELEVIDYS on all endpoints

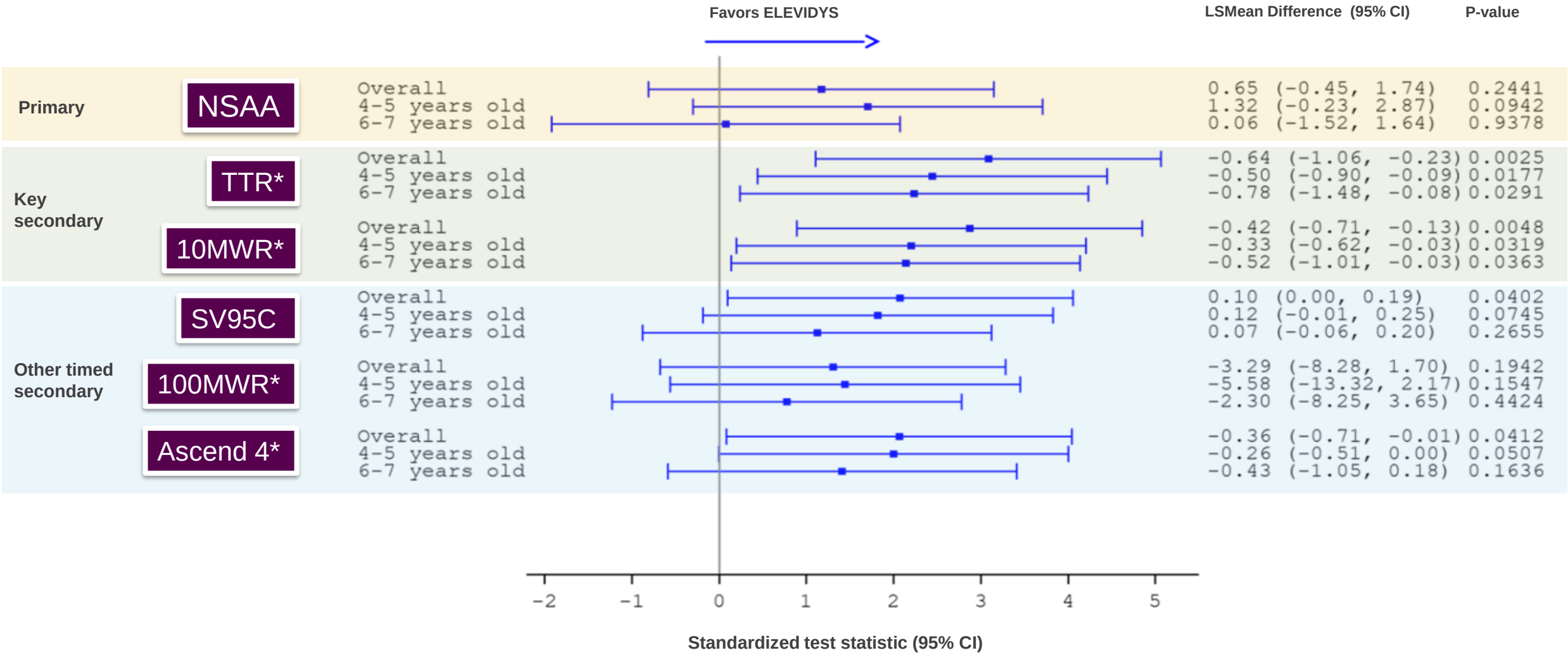
EMBARK achieved statistical significance on all pre-specified key secondary endpoints



* Timed function tests sign reversed to align favorable directions among effect endpoints

** Blue lines plot standardized t test statistic (+/- 1.96) after dividing LSMean (95% CI) by standard error

Functional benefits of ELEVIDYS are not limited to a particular age group



* Timed function tests sign reversed to align favorable directions among effect endpoints
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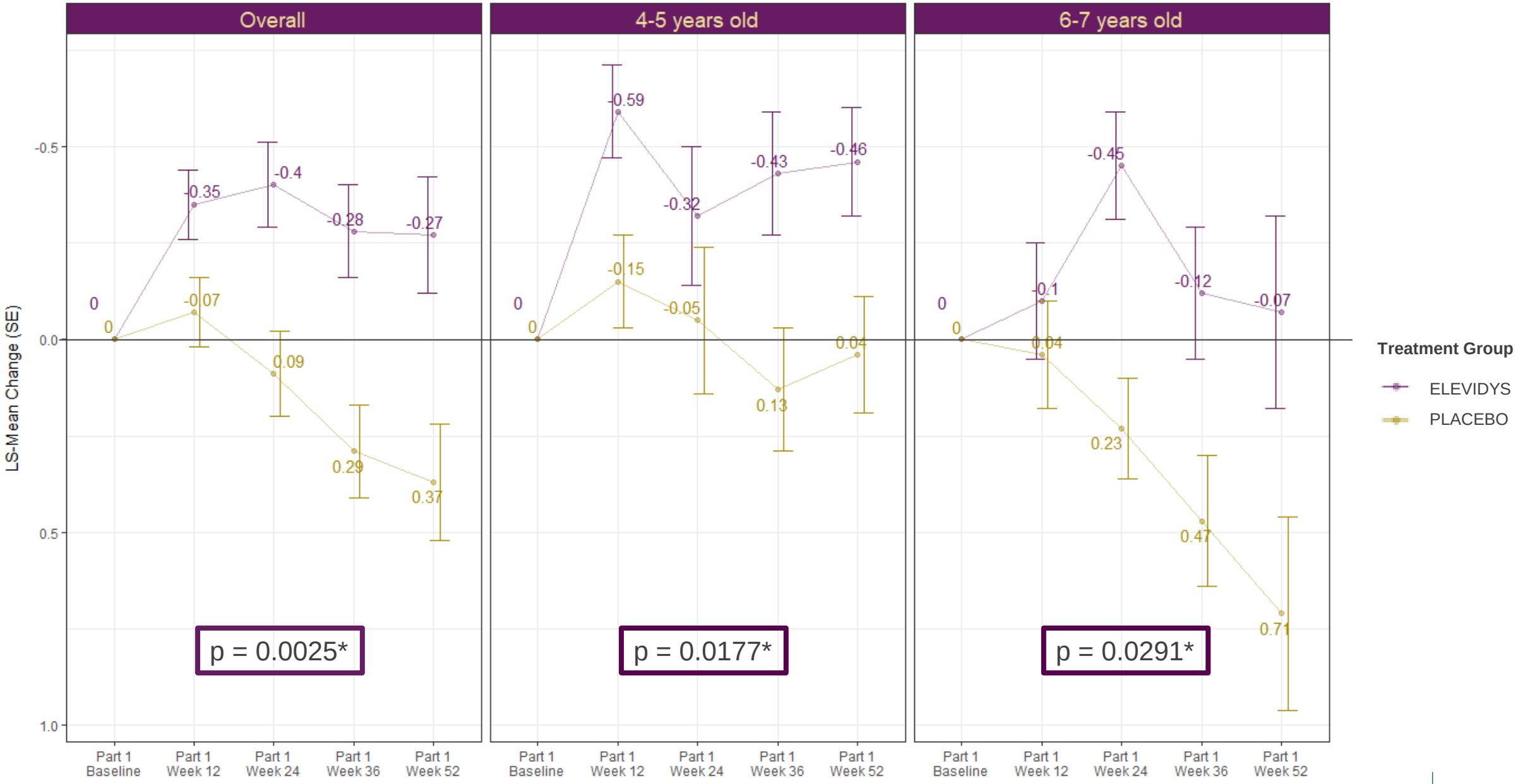
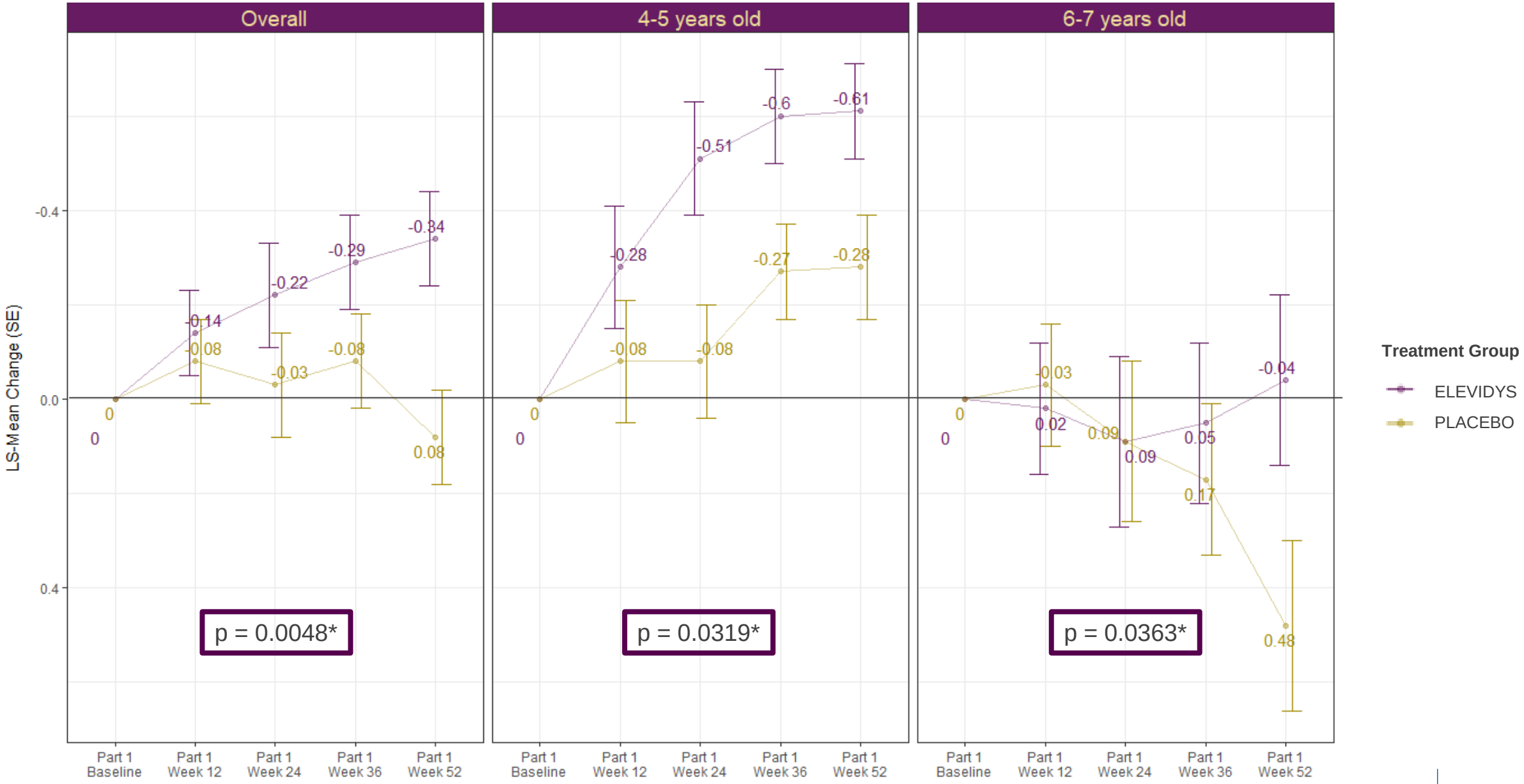
Key Secondary: Rise from floor by visit and age subgroup



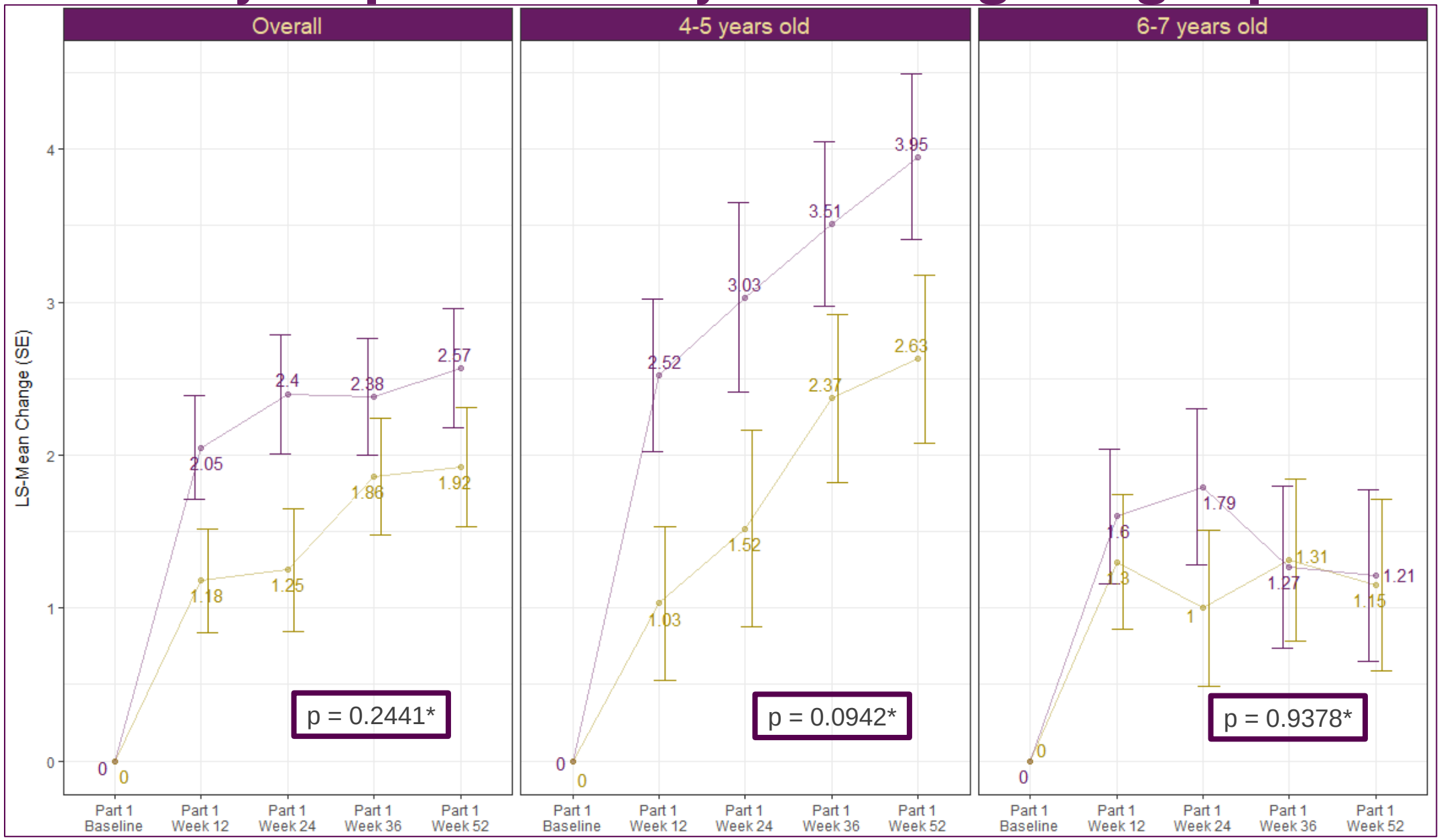
* Comparison at Part 1 Week 52

Key Secondary: 10-meter walk/run by visit and age subgroup



*Comparison at Part 1 Week 52

Primary Endpoint: NSAA by visit and age subgroups



*Comparison at Part 1 Week 52

Safety

Adverse events (AEs) were consistent with safety profile from previous studies

- 125 patients in the safety database
  - 98.4% of patients experienced a TEAE
  - 96.8% of TEAEs were non-serious
- 35.4% of TEAEs were treatment-related
- 4.3% of treatment-related TEAEs were serious
  - 7 (11.1%) patients experienced a treatment-related SAE
- Pattern and severity of AE/SAE was consistent with prior studies
- No complement-mediated adverse events
- No deaths
- No discontinuations

	ELEVIDYS n=63 n (%)	Placebo n=62 n (%)	Total n=125 n (%)
Subjects with any Treatment-Emergent AE (TEAE)	62 (98.4)	57 (91.9)	119 (95.2)
Number of TEAEs	664	502	1166
Number of Treatment-Emergent SAEs	21	9	30
Subjects with any Treatment-related TEAE	48 (76.2)	17 (27.4)	65 (52.0)
Number of Treatment-related TEAEs	235	43	278
Subjects with any Treatment-related SAE	7 (11.1)	0	7 (5.6)
Number of Treatment-related SAEs	10	0	0

Clinical Perspective

Timed tests are responsive to disease change

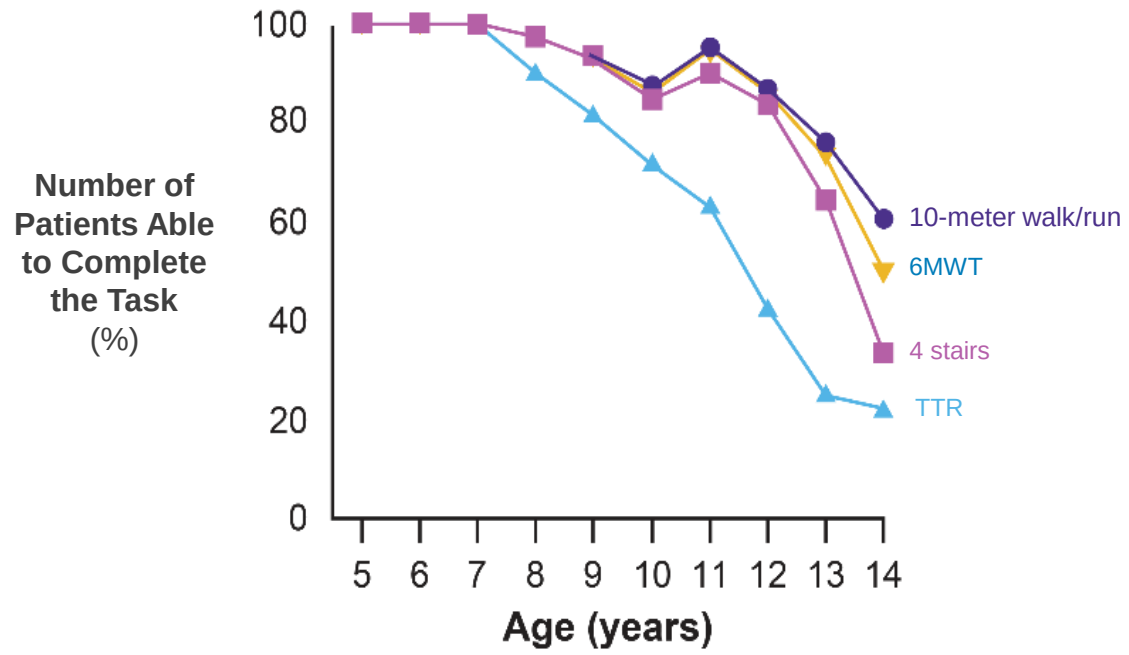
Time to Rise was intentionally selected as the first key secondary functional endpoint:

- Most responsive marker of disease progression in earlier stages of the disease
- Recently used in a registration trial as the primary endpoint
- 5-second rise time is a threshold for predicting disease progression (functional decline)
- Strong prognostic clinical relevance

Time Function Tests are lost in a predictable and sequential manner:

1. Transition to 5-second rise from floor
2. Loss of ability in time to rise
3. Loss of ability to perform the 4-stair climb
4. Loss of ability to perform a 10-meter walk/run

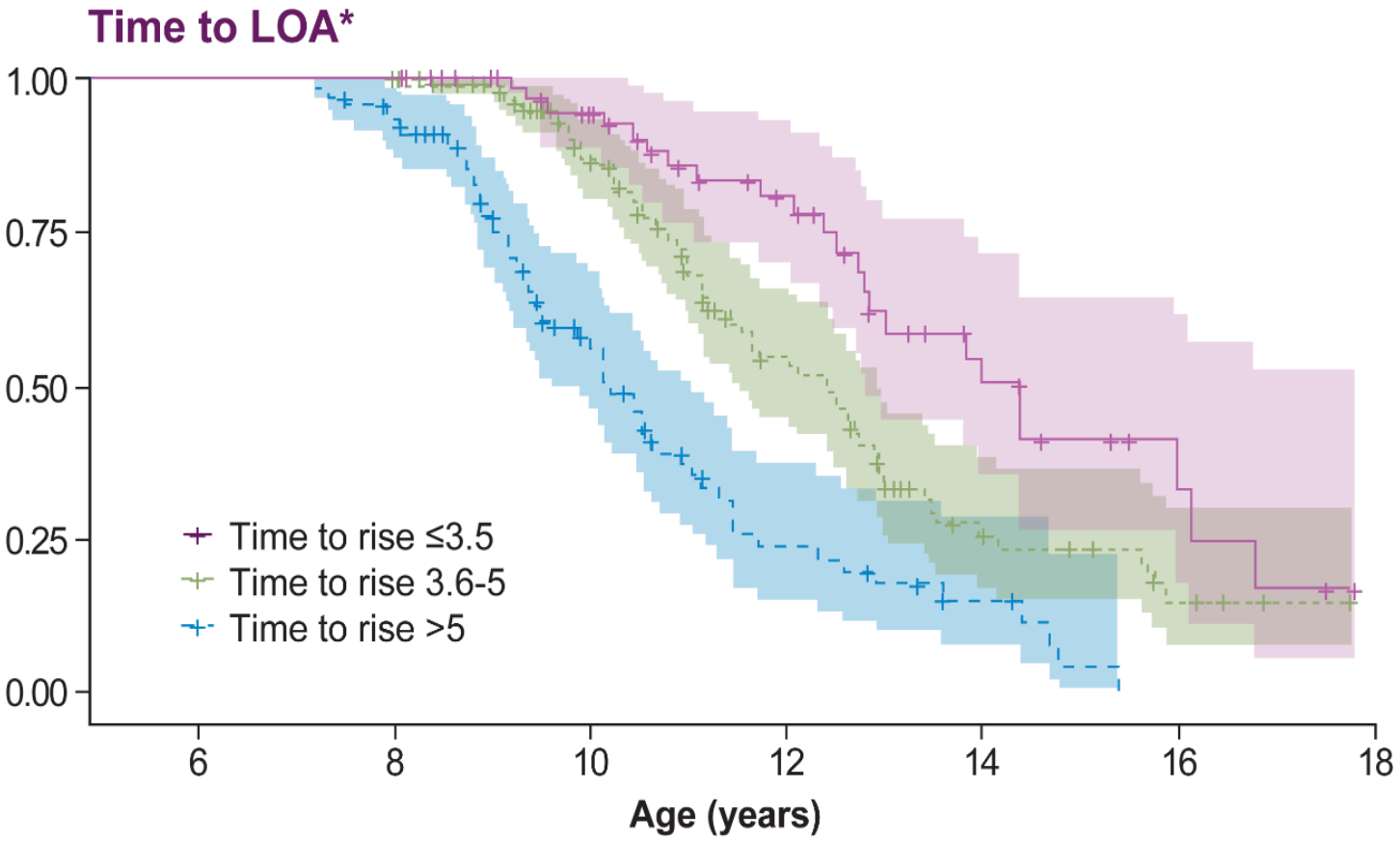
Time Function Tests by Age in Natural History



Sources: McDonald CM. Timed function tests have withstood the test of time as clinically meaningful and responsive endpoints in Duchenne muscular dystrophy. *Muscle Nerve*. 2018 Nov;58(5):614-617. Arora H, Willcocks RJ, Lott DJ, et al. Longitudinal timed function tests in Duchenne muscular dystrophy: ImagingDMD cohort natural history. *Muscle Nerve*. 2018 Nov;58(5):631-638.

Time to Rise is strongly predictive of loss of ambulation

Different durations for Time to Rise predict markedly altered trajectories in time to loss of ambulation according to natural history data



*Total of 293 patients in the UK North Star Network database. LOA = loss of ambulation.
Source: Zambon AA, *et al. Dev Med Child Neurol.* 2022;64(8):979-88.

Treatment with ELEVIDYS reduced the odds of progressing to a rise time of greater than 5 seconds by 91%

Age Group	% Patient > 5 sec at W52		% Reduction in odds
	ELEVIDYS	Placebo	
Overall	3%	16%	**91% (p=0.0135)**
6-7 Years	6%	25%	**89% (p=0.0335)**

Path Forward & Summary

Path forward

ELEVIDYS trials remain ongoing as we pursue label expansion

- Provided topline summary of results to FDA
- Engaged in productive conversations and feedback on the data
- Plan to submit efficacy supplement to CBER as soon as possible seeking label expansion to treat all patients with Duchenne
- Plan to submit postmarketing requirement (PMR) seeking transition from accelerated approval to traditional approval
- Full EMBARK results to be presented and published in future scientific forums

Summary

- The data from EMBARK exceed the threshold for substantial evidence of effectiveness and the risk/benefit of ELEVIDYS remains favorable
- We are pleased with the consistency, the magnitude of response and the clinical meaningfulness of the results from EMBARK and from the body of evidence supporting ELEVIDYS
- The data support ELEVIDYS as a disease-modifying therapy and therefore we believe all patients with Duchenne can benefit from treatment
- Following positive discussions with FDA leadership, they are committed to evaluating a labeling expansion to the fullest extent possible based on a review of the data and will do so rapidly

Q&A

Closing Remarks

Patients can’t wait for the next breakthrough in medical research.

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