

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

**So neither will we.**

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*Chief Scientific Officer and Head of R&D*

EMBARK Part 2 Data

January 27, 2025



**BENJAMIN**

Living with Duchenne  
muscular dystrophy

# 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 statements relating to ELEVIDYS and our SRP-9001 trials; our future operations; potential solutions and market opportunities with our technologies and those with our strategic partners; and expected plans and milestones, including presenting and publishing data in scientific forums.

These forward-looking statements involve risks and uncertainties, many of which are beyond our control and are based on our current beliefs, expectations and assumptions regarding our business. Actual results and financial condition could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties, and such risks and uncertainties could materially and adversely affect our business, results of operations and trading price. Potential known risk factors include, among others, the following: the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; the results of future research may not be consistent with past positive results; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; our dependence on our manufacturers to fulfill our needs for our clinical trials and commercial supply, including any failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of products to successfully support various programs; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, and clinical development plans for various reasons, including possible limitations of our 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; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) and in its other SEC filings.

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# EMBARK

**(SRP-9001-301) Part 2 Results**

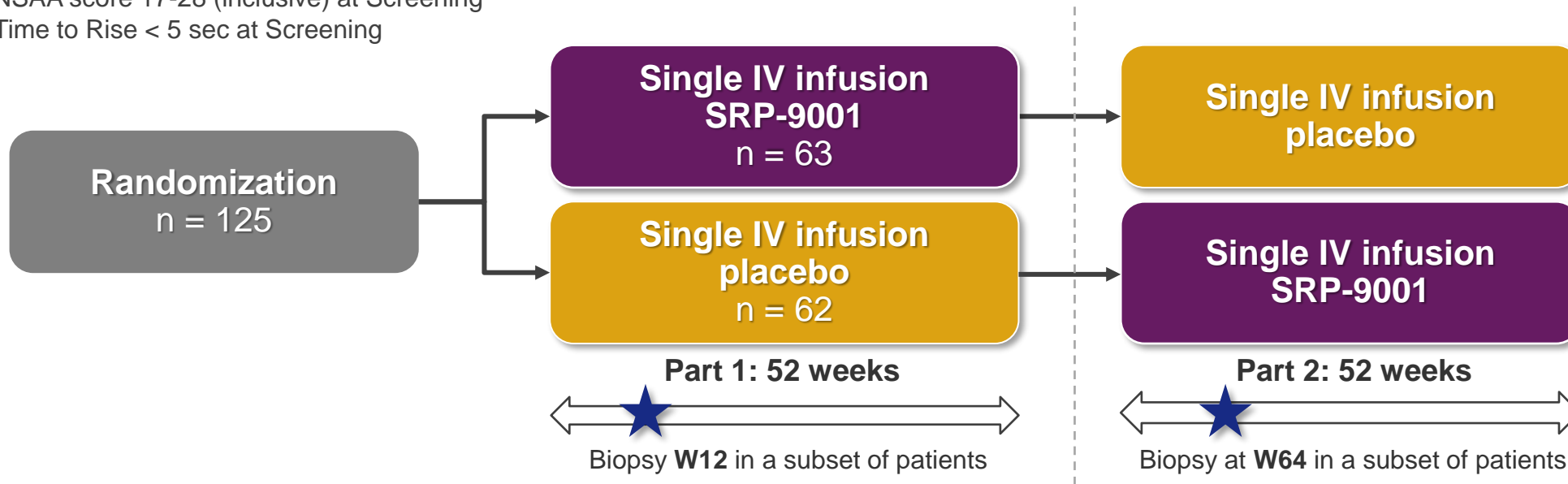
# EMBARK (Study SRP-9001-301) Study Design



Phase 3, multinational, double-blind, randomized, placebo-controlled study evaluating the safety and efficacy of ELEVIDYS (delandistrogene moxeparvovec), 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

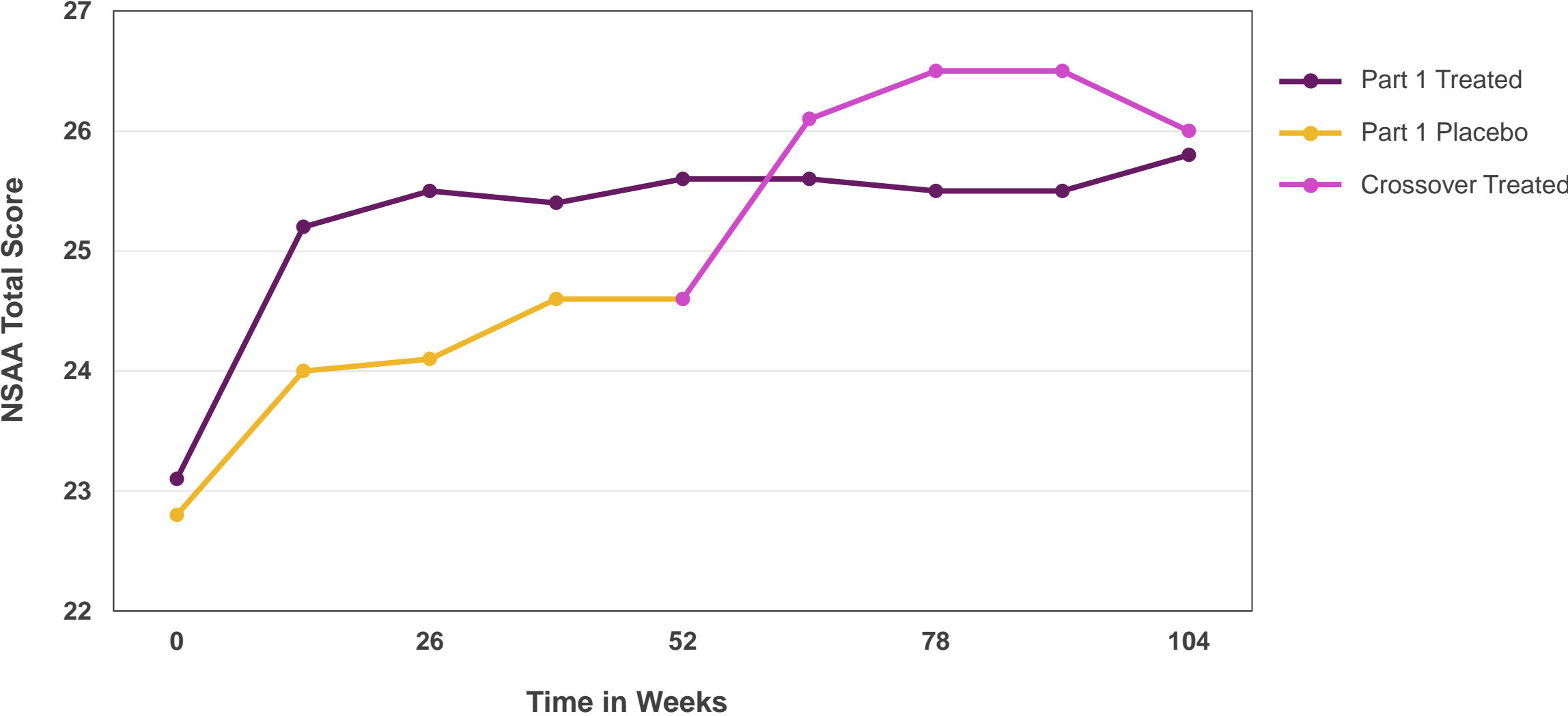
## Key Secondary endpoints:

- 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:

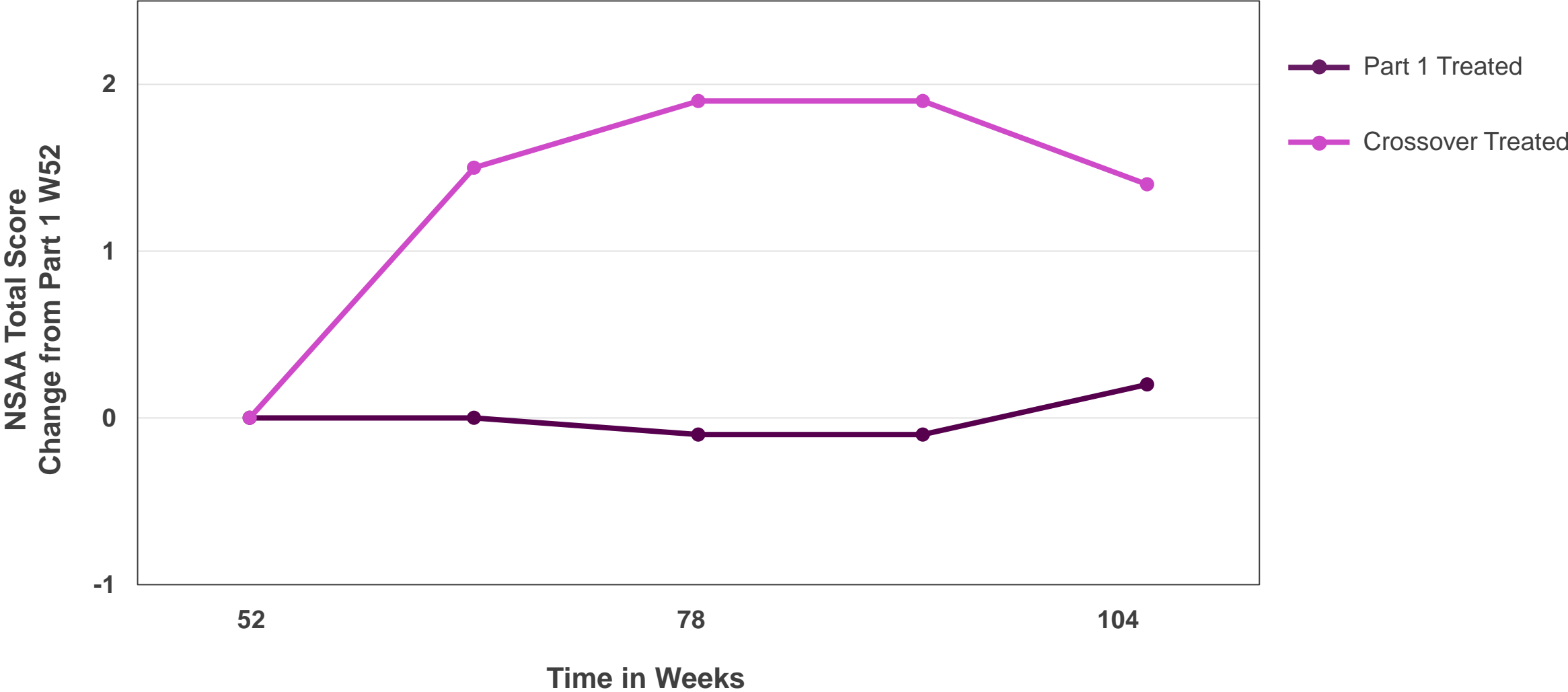
- Stride Velocity 95th Centile (SV95C)
- 100-meter walk/run
- Ascend 4 Steps

# EMBARC NSAAS\* Total Score: Baseline to Year 2, blinded study results



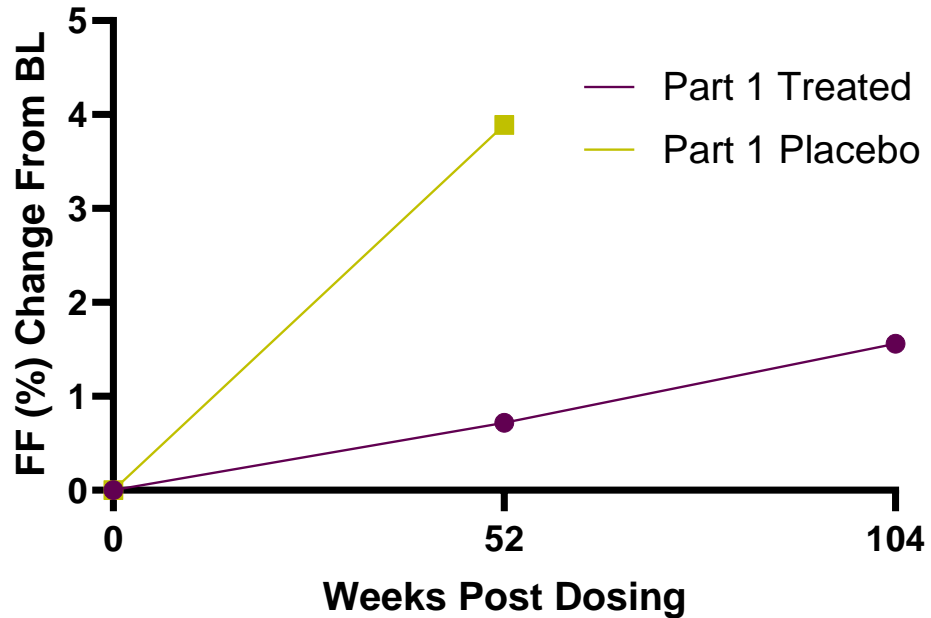
\*North Star Ambulatory Assessment

# NSAA increases in crossover treated patients following ELEVIDYS dosing



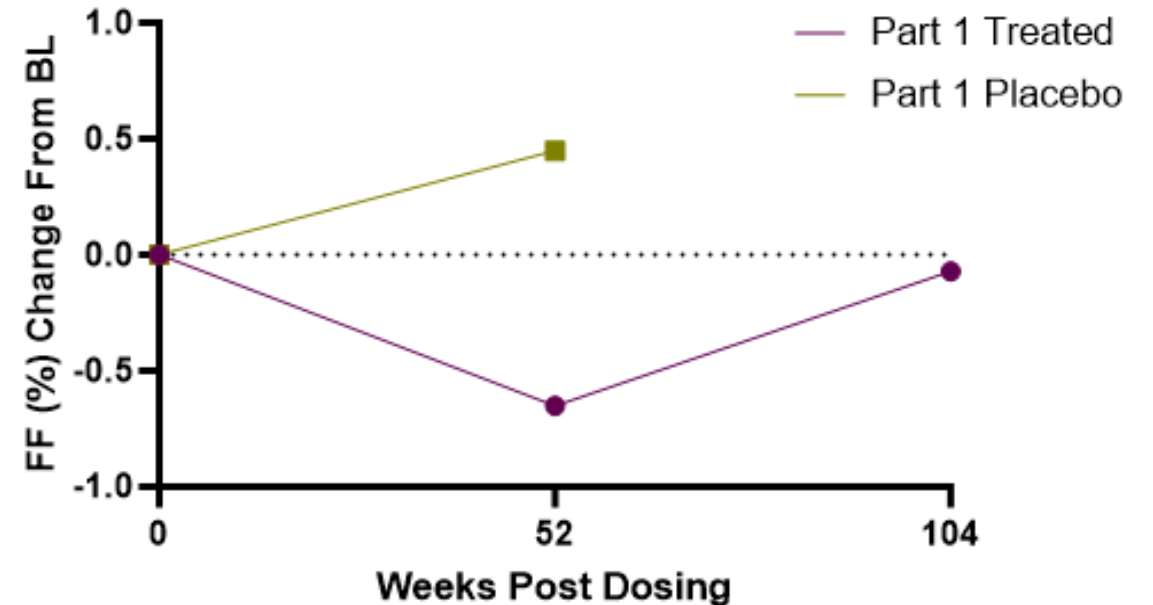
# 2 Year Results: Musculoskeletal MRI - Fat Fraction

## Vastus Lateralis



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

## FF Soleus

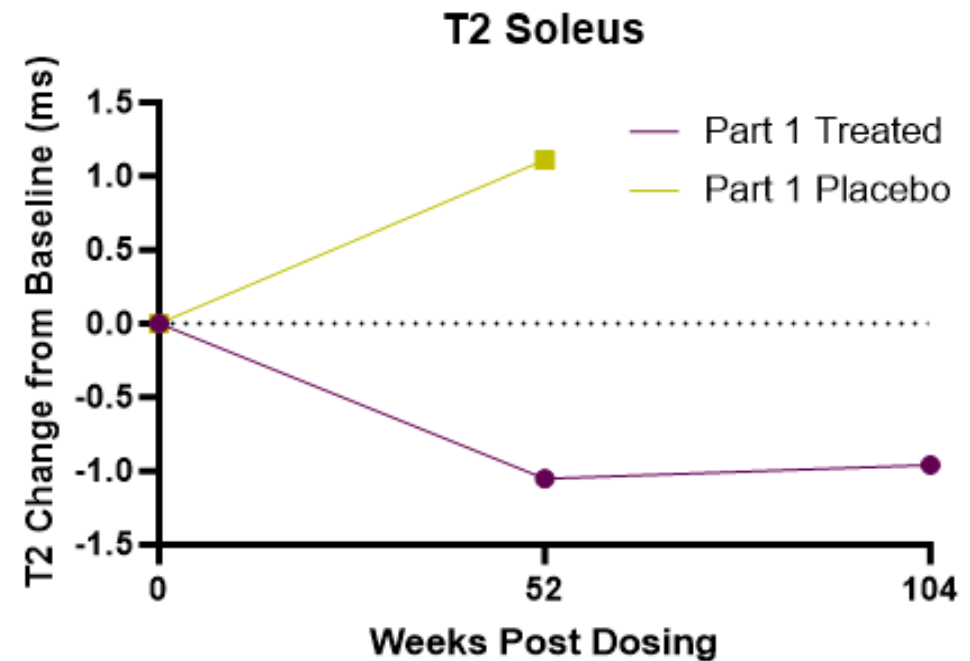
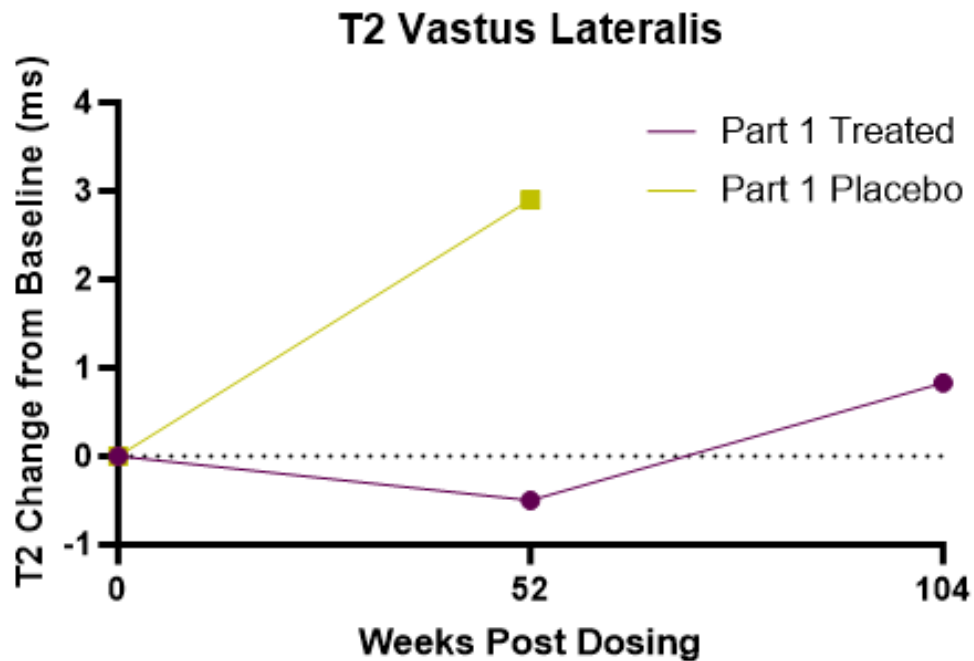


N=17	N=15	
N=16	N=16	N=15

**Stabilization in MRI muscle FF in 9001 treatment group with two years values below those seen in part 1 placebo group**

1. Naarding KJ, Reyngoudt H, van Zwet EW, et al. 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, et al. MR biomarkers predict clinical function in Duchenne muscular dystrophy. *Neurology*. 2020 Mar 3;94(9):e897-e909.
3. Willcocks RJ, Rooney WD, Triplett WT, et al. 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.

# 2 Year Results: Musculoskeletal MRI - T2



Placebo N=18  
Treated N=13

N=15  
N=12

N=12

N=17  
N=15

N=15  
N=15

N=14

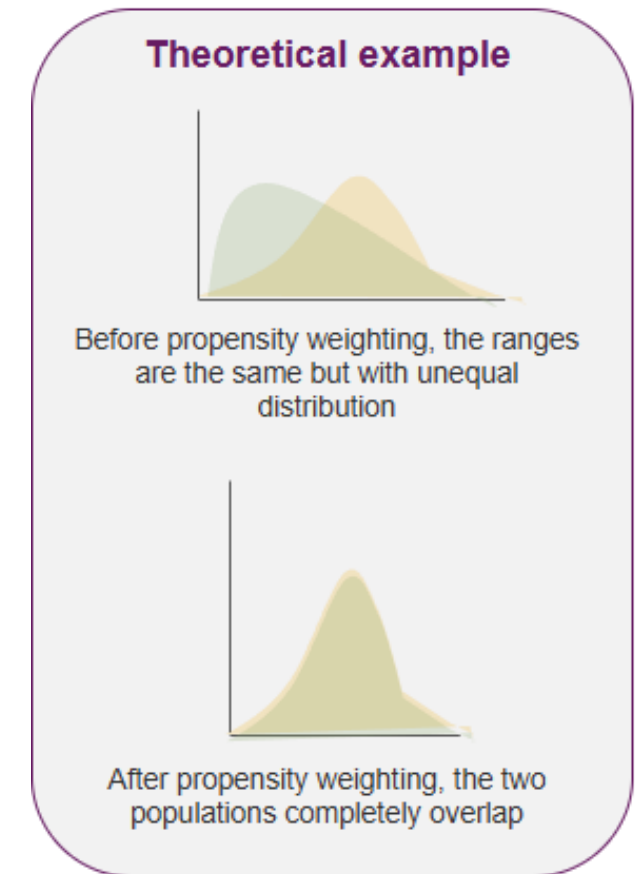
**Stable muscle T2 in most muscle groups over 2 years with lower T2 2 years after Elevidys treatment than at 1 year in placebo cohort**

1. Naarding KJ, Reyngoudt H, van Zwet EW, et al. 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, et al. MR biomarkers predict clinical function in Duchenne muscular dystrophy. *Neurology*. 2020 Mar 3;94(9):e897-e909.
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# **EMBARC Part 2 Results: Crossover-treated patients compared to external control, 1 Year Data**

# EMBARK Statistical Analysis Plan and External Control

- 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
  - DEMAND-III placebo arm
  - PRO-DMD
  - CINRG DNHS
  - FOR-DMD
- Propensity score weighting is used to decrease bias
- Protocols agreed to by regulatory authorities prior to the start of the study

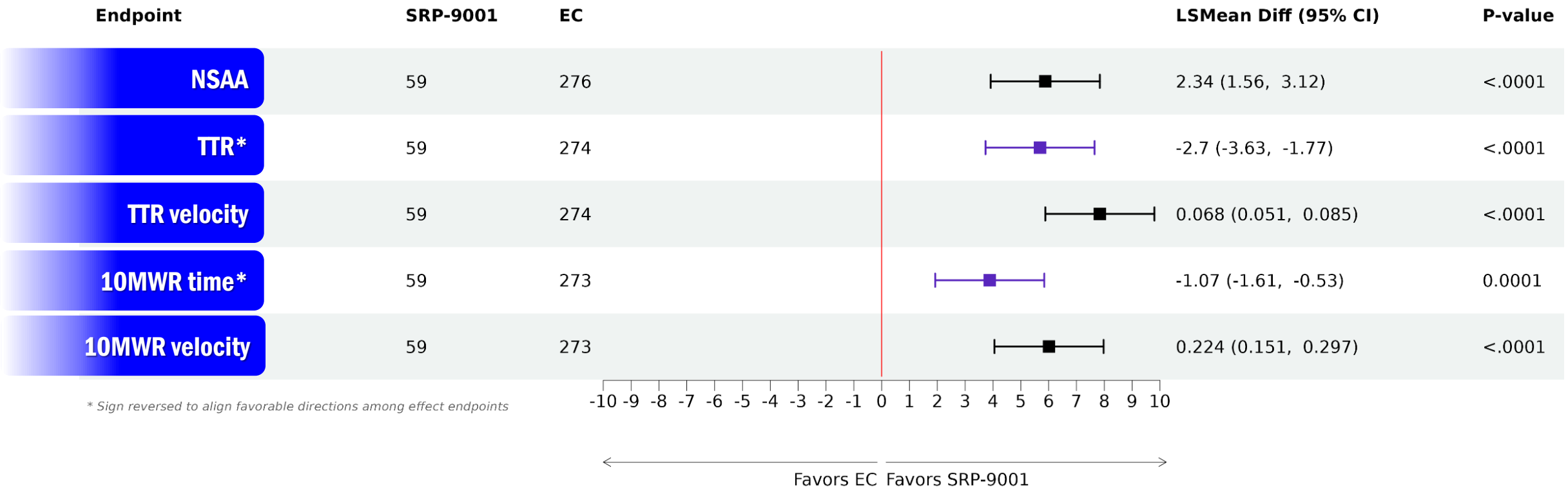


# Balanced baseline characteristics for crossover-treated patients and external control (EC)

**Baseline Characteristics (Weighted)**  
Full Analysis Set

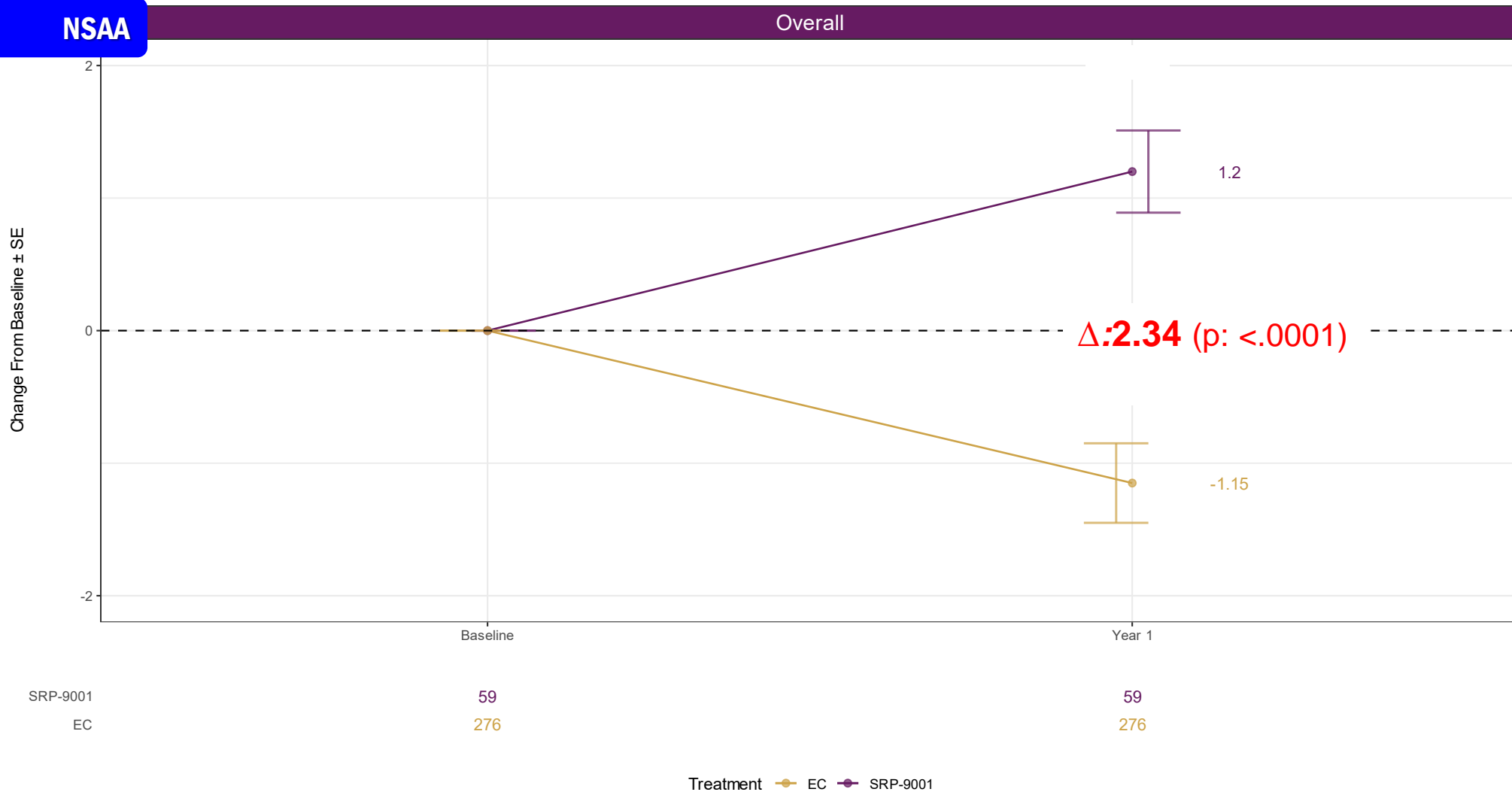
<b>Baseline</b>	<b>SRP-9001 (n=59)</b> Mean (Min, Max)	<b>External Control (n=276)</b> Mean (Min, Max)
<b>Age</b>	7.11 (5.07, 9.07)	7.07 (5.05, 9.98)
<b>NSAA</b>	25.0 (12, 34)	25.0 (12, 34)
<b>Time to Rise from Floor</b>	4.01 (2.00, 11.75)	3.94 (1.60, 10.60)
<b>10M Walk/Run Time</b>	5.01 (3.30, 10.90)	4.99 (2.70, 10.20)
<b>Weight</b>	25.52 (14.9, 49.9)	24.45 (14.7, 42.4)
<b>Height</b>	114.36 (97.7, 131.0)	113.85 (94.9, 142.0)
<b>BMI</b>	19.17 (13.98, 35.54)	18.61 (13.74, 25.88)

# Statistically significant and clinically meaningful improvements in functional outcomes one year after treatment compared to EC

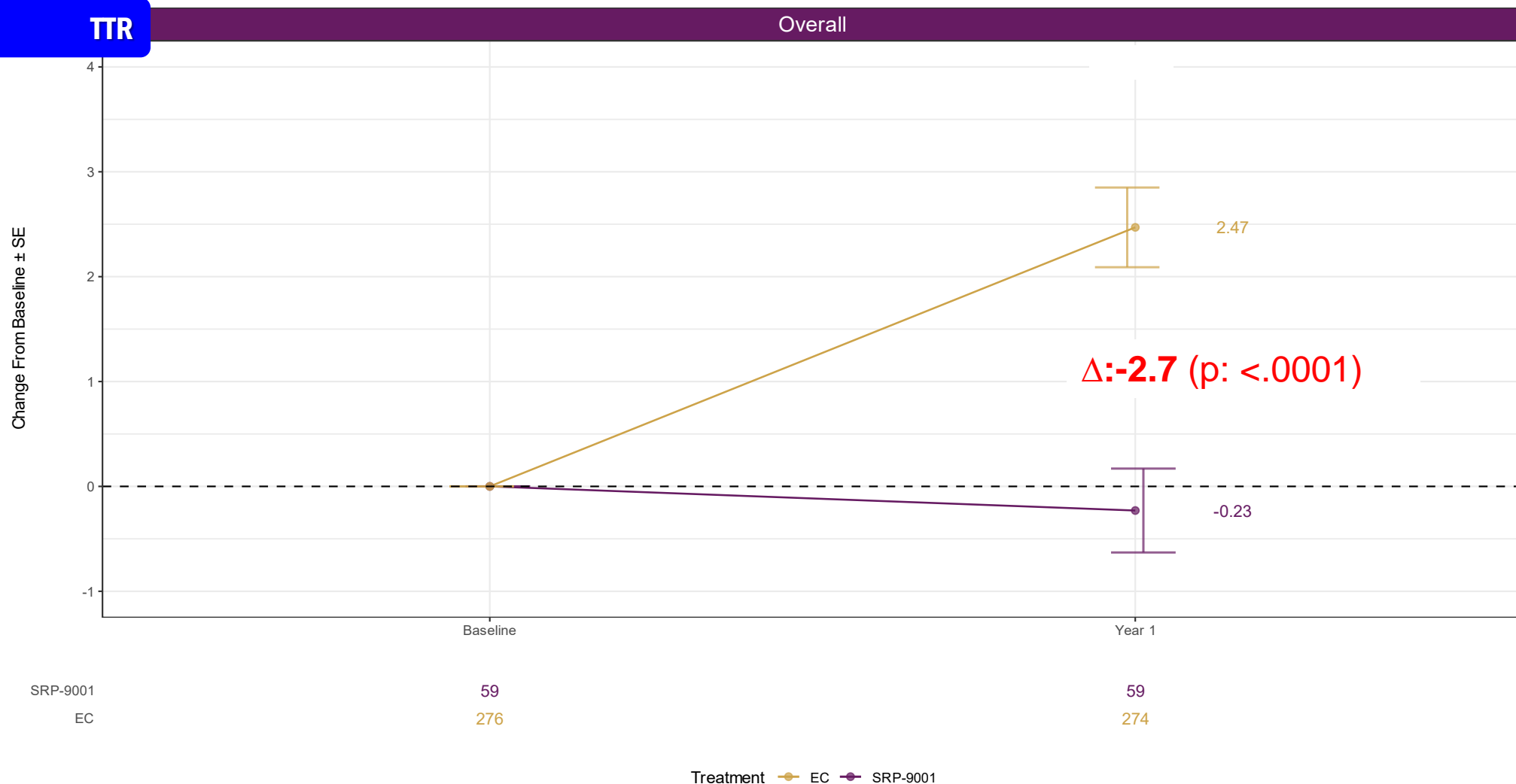


Diff in Means (of change from baseline) and confidence intervals are standardized by dividing by the standard error (SE). The half width of the forest plot bars will be 1.96. Numerical results of the Diff in Means are on original scale (without SE adjustment) along with P-values (unadjusted nominal).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

# Statistically significant increase in NSAA one year after treatment compared to EC



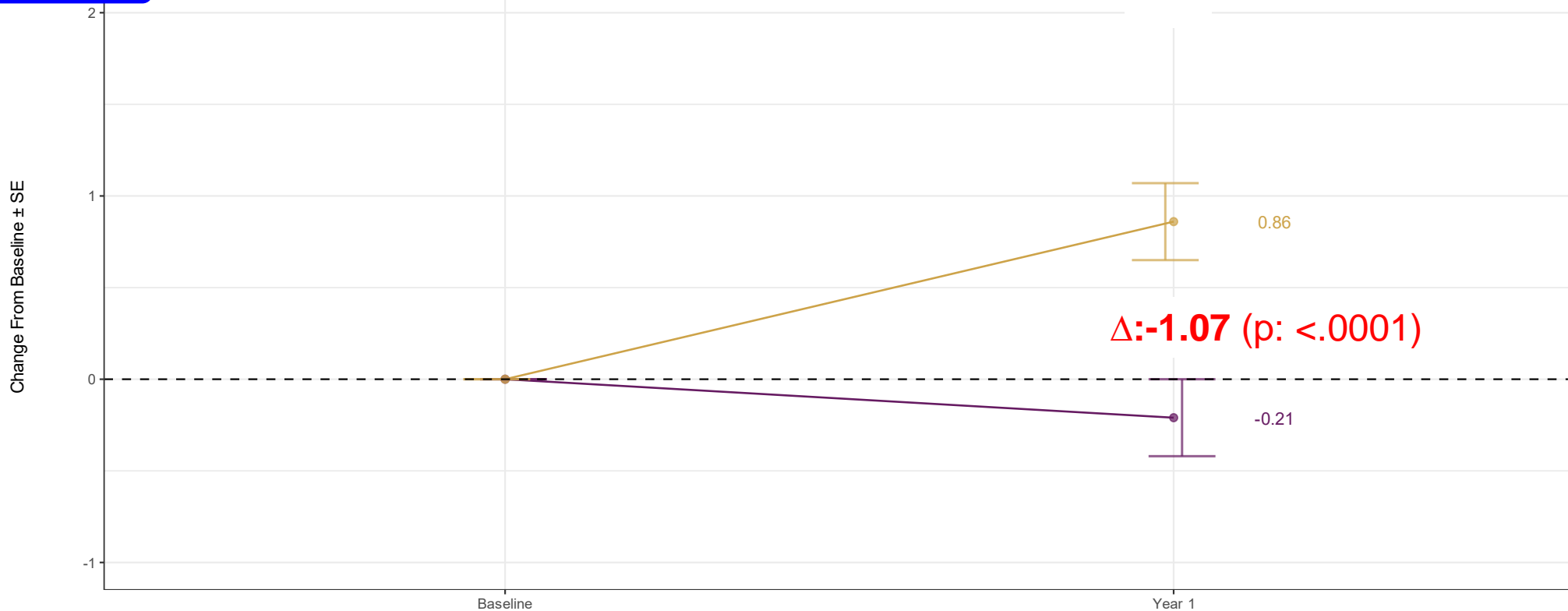
# Statistically significant improvements in time-to-rise from floor one year after treatment compared to EC



# Statistically significant improvement in 10m walk/run times one year after treatment compared to EC

10MWR time

Overall



SRP-9001  
EC

59  
276

59  
273

Treatment — EC — SRP-9001

# **EMBARC Part 2 Results: Year 2 Expression and Functional outcomes in Part-1 treated patients**

# Expression is consistent and sustained from week 12 to week 64

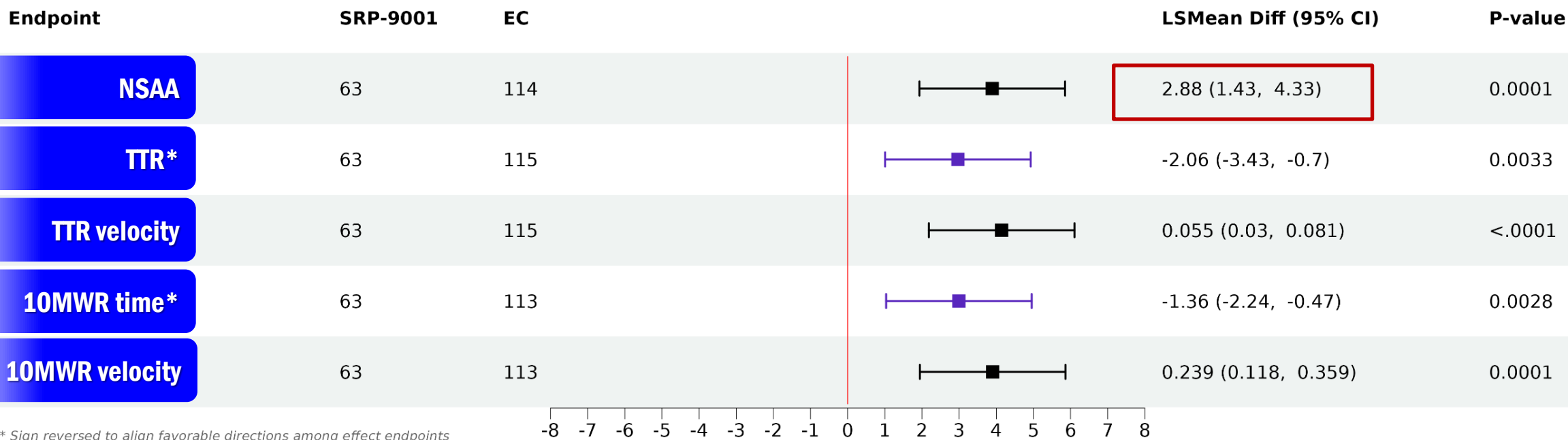
Western Blot	Part 1 SRP-treated	
	Week-12 n = 17	Week-64 n = 16
Mean (SD)	<b>34.29 (41.04)</b>	<b>45.68 (39.75)</b>

# Balanced Baseline Characteristics for Part 1 Treated and EC

## Baseline Characteristics (Weighted) Full Analysis Set

Baseline	SRP-9001 (n=64) Mean (Min, Max)	External Control (n=143) 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 from Floor	3.51 (1.85, 5.75)	3.52 (1.90, 5.70)
10M Walk/Run Time	4.80 (3.20, 6.85)	4.78 (3.00, 6.70)
Weight	21.20 (13.5, 37.4)	22.18 (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)

# Statistically significant and clinically meaningful differences after treatment at Year 2 compared to EC

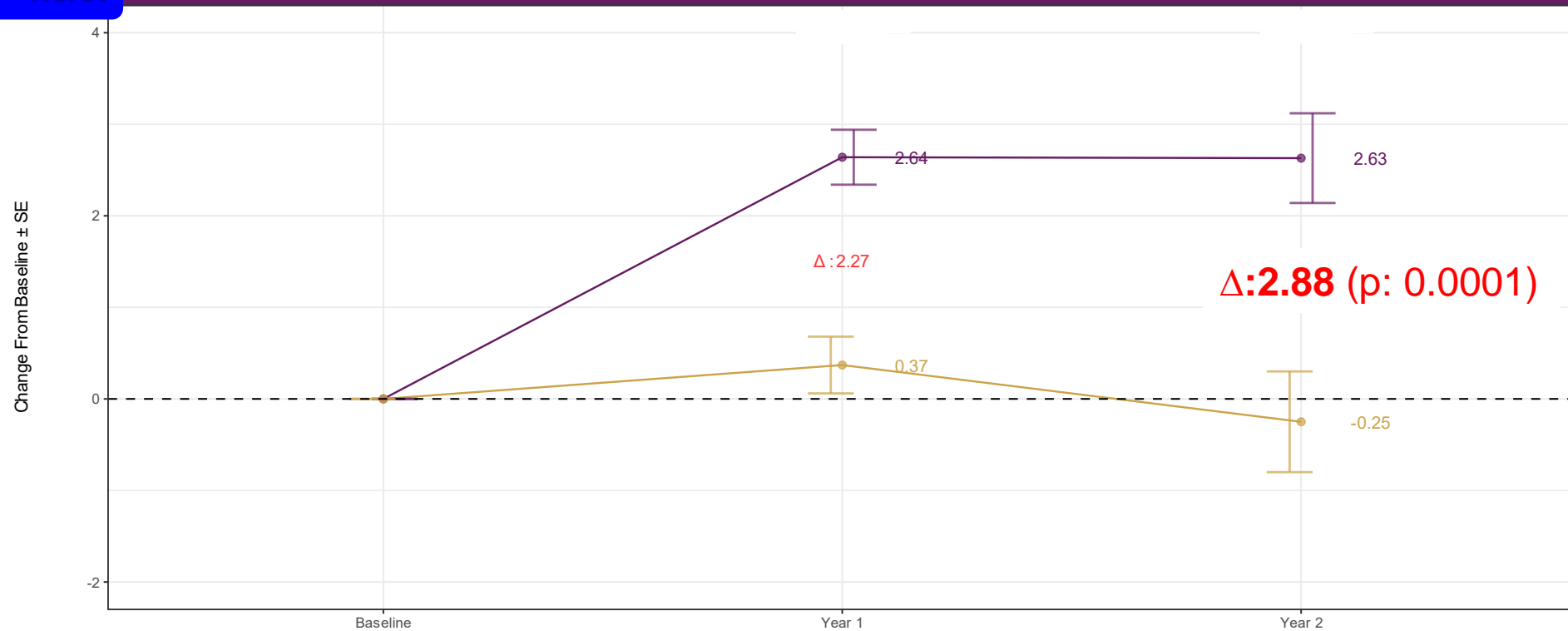


Diff in Means (of change from baseline) and confidence intervals are standardized by dividing by the standard error (SE). The half width of the forest plot bars will be 1.96. Numerical results of the Diff in Means are on original scale (without SE adjustment) along with P-values (unadjusted nominal).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

# NSAA improvements were sustained two years after treatment, showing a widening divergence from natural history

NSAA

Overall



SRP-9001  
EC

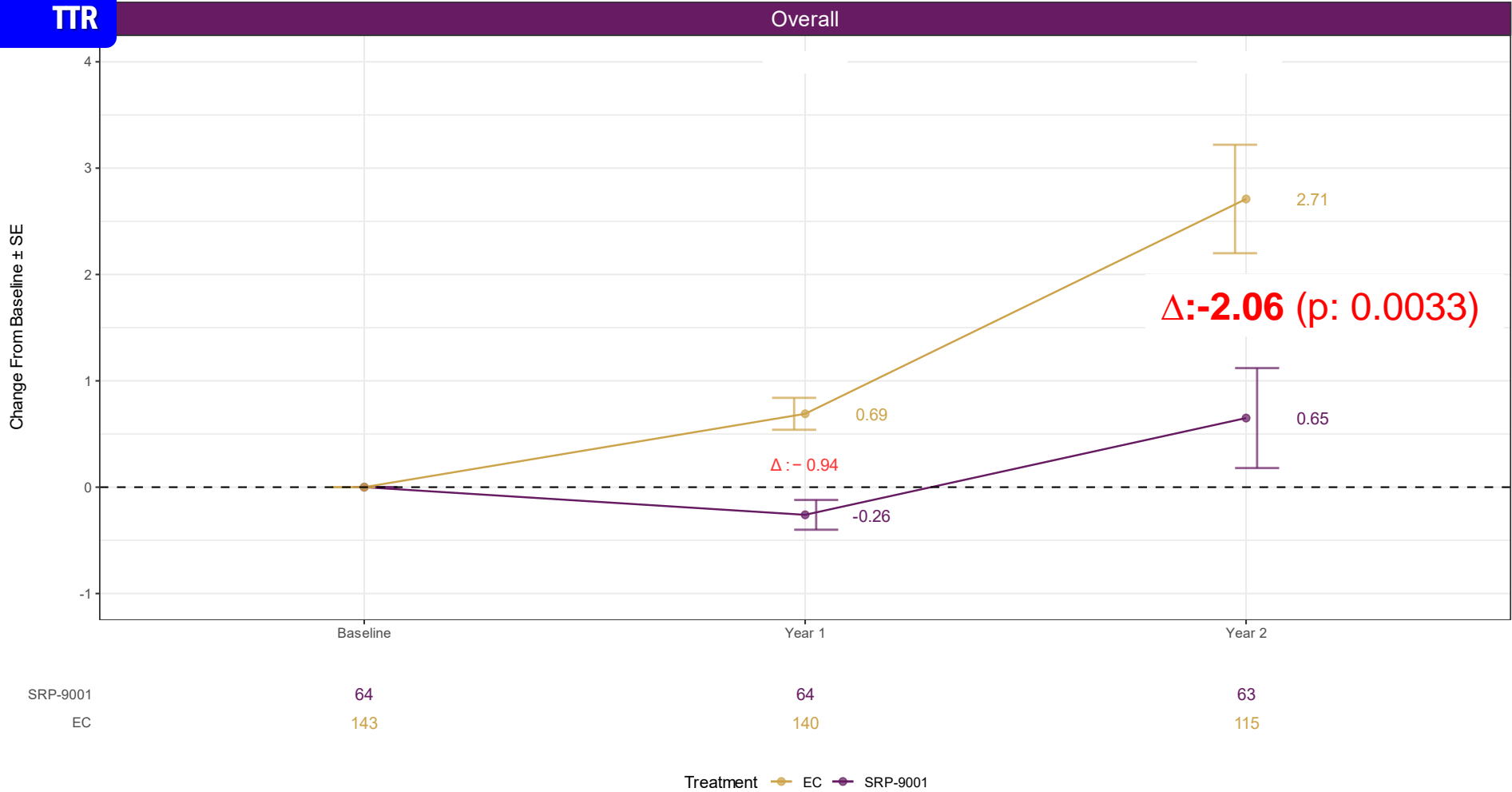
64  
143

64  
141

63  
114

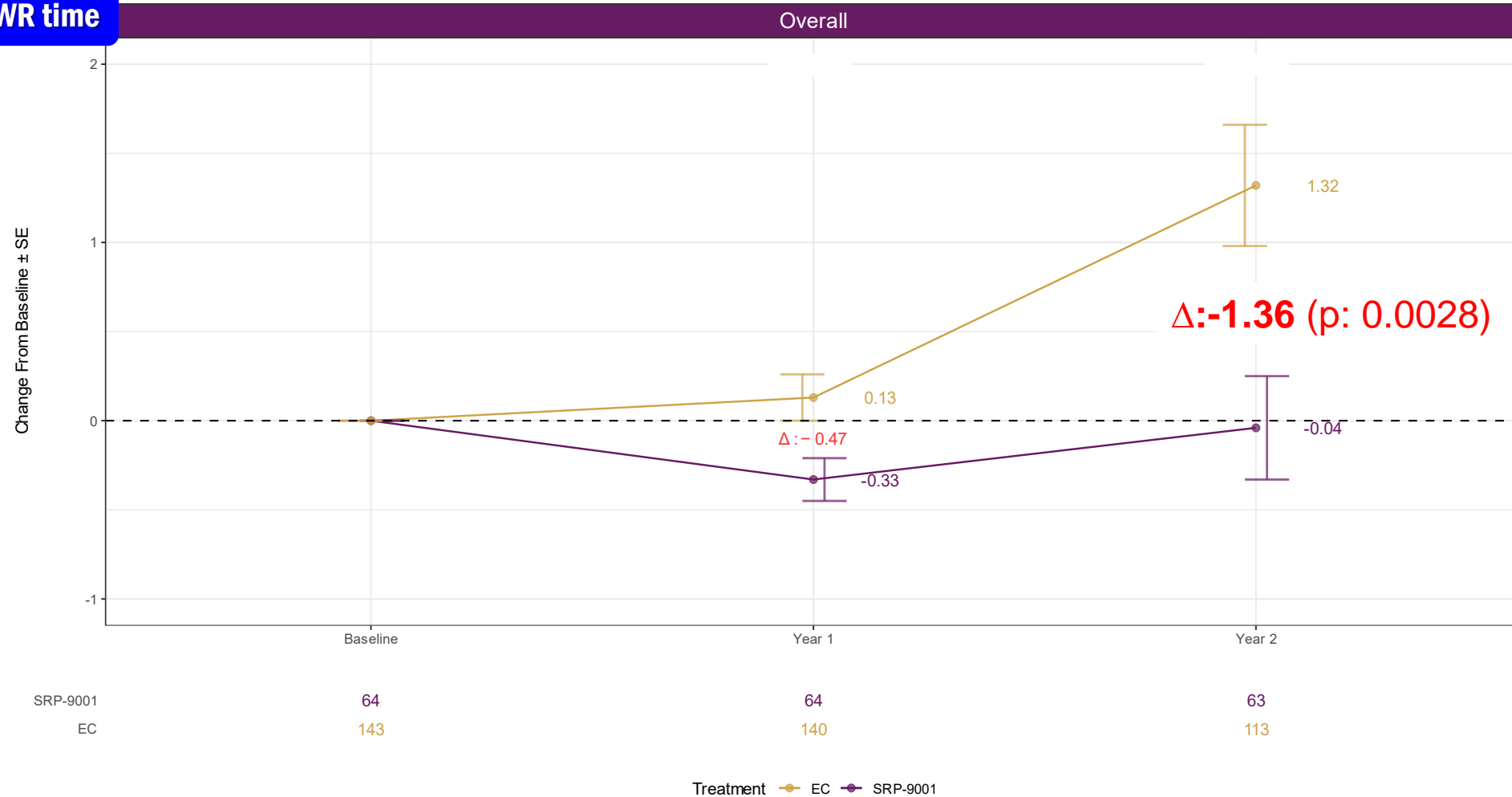
Treatment — EC — SRP-9001

# Time-to-rise over two years also demonstrates a widening divergence over time



# 10MWR remains stable two years after treatment, with a widening divergence from natural history over time

10MWR time



# Safety Data

# Safety profile in EMBARK is consistent with previous studies

	Part 1 Treated, Year 1 post-treatment N=63 n (%)	Part 1 Treated, Year 2 post-treatment** N=63 n (%)	Crossover Treated, Year 1 post-treatment N=60 n (%)	Total SRP-9001 Treated* N=123 n (%)
TEAE	62 ( 98.4)	0	56 ( 93.3)	118 ( 95.9)
SAEs	14 ( 22.2)	1 (1.6)	8 ( 13.3)	23 ( 18.7)
Treatment-related TEAE	48 ( 76.2)	4 ( 6.3)	50 ( 83.3)	102 ( 82.9)
Treatment-related SAEs	7 ( 11.1)	0	7 ( 11.7)	14 ( 11.4)
AEs leading to study discontinuation	0	0	0	0
Deaths	0	0	0	0

\*Total of 123 patients received SRP-9001 in EMBARK Part 1 and 2.

\*\*Patients who had not experienced corresponding event in part-1 but had in part-2.

# No safety concerns on cardiac MRI over 2 years of follow-up

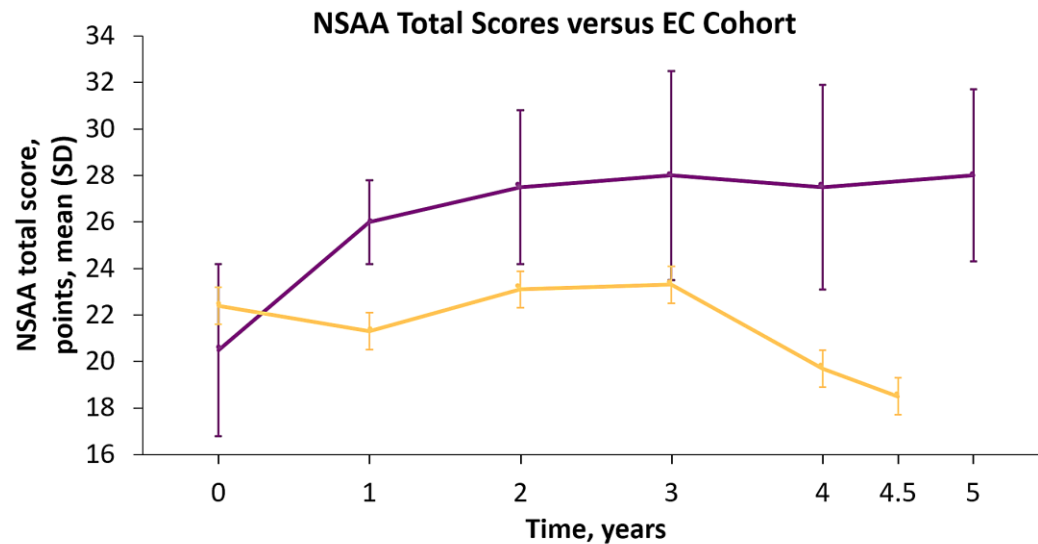
- Values within normal range
- No statistical or clinical differences in these groups

	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)

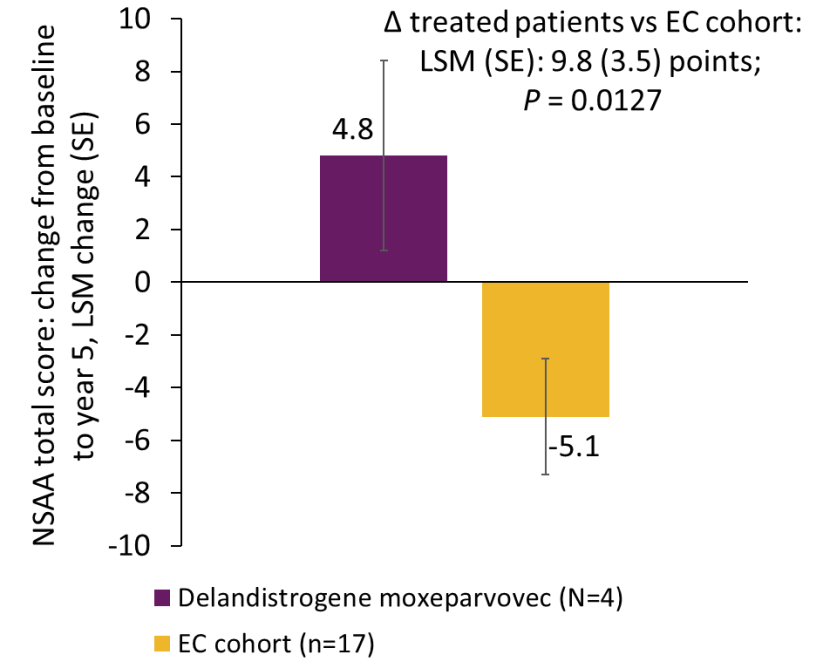
# Summary & Next Steps

# Study 9001-101 Year 5 Results Demonstrate Durable Response

*9.8-point difference on mean NSAA in patients receiving SRP-9001 compared to external control group*



Delandistrogene moxeparvovec	4	4	4	4	4	4
EC cohort	17	17	17	17	17	17



SRP-9001 treated patients showed a sustained increase in NSAA total score over 5 years, with a statistically significant and clinically meaningful difference at year 5 compared with the EC cohort.

At 5 years post-infusion, the mean (range) age of patients was 10.14 (9.02–11.02) years

1. Balancing for age was limited by a reduced number of suitable patients in the external control database with 5-year functional data. Groups are well balanced for functional assessments predictive of disease progression.\*NSAA change from baseline over 5 years SRP-9001 vs External Control calculated using unadjusted means. BL, baseline; EC, external control; LSM, least square mean; NSAA, North Star Ambulatory Assessment; SD, standard deviation; SE, standard error.

# Summary and Next Steps

- Treatment with ELEVIDYS corresponded with increases on the NSAA at one year in crossover patients, while the study remained blinded
- Statistically significant increases on NSAA, TTR and 10MWR when compared to a pre-specified, well-matched external controls for crossover-treated patients one year after treatment
- Results from Part 1-treated patients demonstrate benefits and disease stabilization two years after treatment
  - Across NSAA, TTR and 10MWR results in treated patients were statistically significant two years post-dosing compared to a pre-specified, propensity-weighted external control group.
  - Expression is consistent with data from other ELEVIDYS clinical trials and sustained week 12 to week 64 after treatment
  - MRI continues to show minimal progression in underlying muscle pathology and remains consistent with the functional benefits shown
- Benefit-risk profile remains favorable; no new safety signals were observed
- EMBARK Part 2 results add meaningfully to the totality of evidence supporting treatment with ELEVIDYS and support the conclusion that ELEVIDYS modifies the trajectory of Duchenne
- Long-term follow up of patients will continue and we will work to present and publish data in scientific forums

# Q&A



# EMBARK

**(SRP-9001-301) Part 2 Results**