SRP-9001: New Clinical Data and Integrated Analysis

DOUG INGRAM
President and Chief Executive Officer

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July 6, 2022
8:30 a.m. ET

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 the potential benefits of our technologies and scientific approaches; the estimated number of patients suffering from Duchenne; the potential benefits of SRP-9001, including the potential benefits of our SRP-9001 gene therapy construct, our belief that SRP-9001 has a safety profile that is consistent and manageable, and the potential for SRP-9001 to alter the trajectory of the disease; our ongoing global Phase 3 EMBARK Study; our goal to change the course of Duchenne; and expected milestones and plans, including completing ongoing regulatory discussions related to SRP-9001 and then providing an update and completing enrollment in EMBARK in the middle of the year.

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: success in preclinical trials and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; different methodologies, assumptions and applications we utilize 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; if the actual number of patients living with Duchenne is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, due to a variety of reasons, some 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 the ongoing COVID-19 pandemic; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2021, and 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.

For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's filings with the SEC. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. The forward-looking statements in this presentation are made as of the date of this presentation only and, other than as required under applicable law, Sarepta does not undertake any obligation to publicly update its forward-looking statements.

Agenda

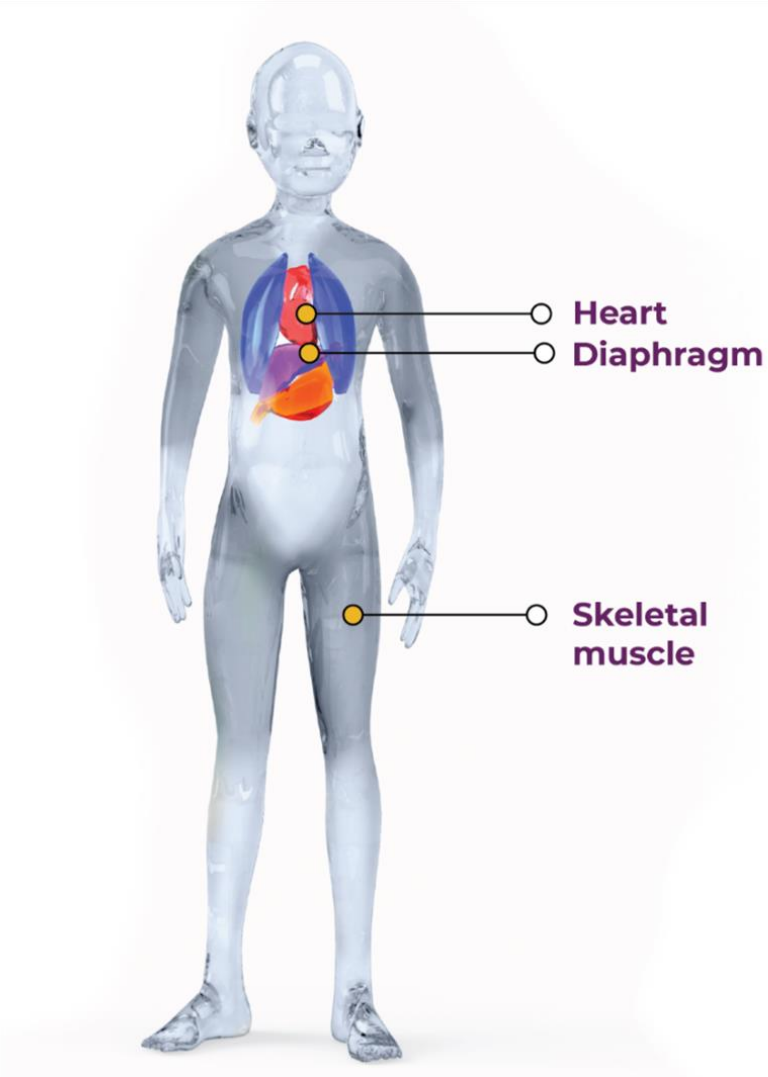
- **New Clinical Evidence**
  - SRP-9001-103 (ENDEAVOR), Cohort 1 (n=20): 1-year functional data (from baseline and compared to external control)
  - Long-term Data
    - SRP-9001-101 (n=4): 4-year follow-up data (NSAA and timed function tests)
    - SRP-9001-102, Part 1 (n=20): 2-year functional data (n=19)*
  - Integrated Efficacy Analysis, SRP-9001-101, 102, 103 (n=52) at target dose: 1-year functional data (compared to propensity-weighted control)
- **Expression Data**
- **Safety**
- **Summary & Next Steps**
- **Q&A**

*20 patients received active therapy in Part 1, one of these patients did not have an in-person assessment at Part 2 Week 48.

Duchenne Muscular Dystrophy (Duchenne)

Duchenne affects approximately 1 in 3,500-5,000 newborn males worldwide[1]

- Duchenne is a rare, fatal neuromuscular genetic disease inherited in an X-linked recessive pattern[2]
- Muscle weakness becomes increasingly noticeable by 3 to 5 years of age, and most patients use a wheelchair by the time they are 10 to 14 years old[2]
- During adolescence, cardiac and respiratory muscle deterioration lead to serious, life-threatening complications[3]

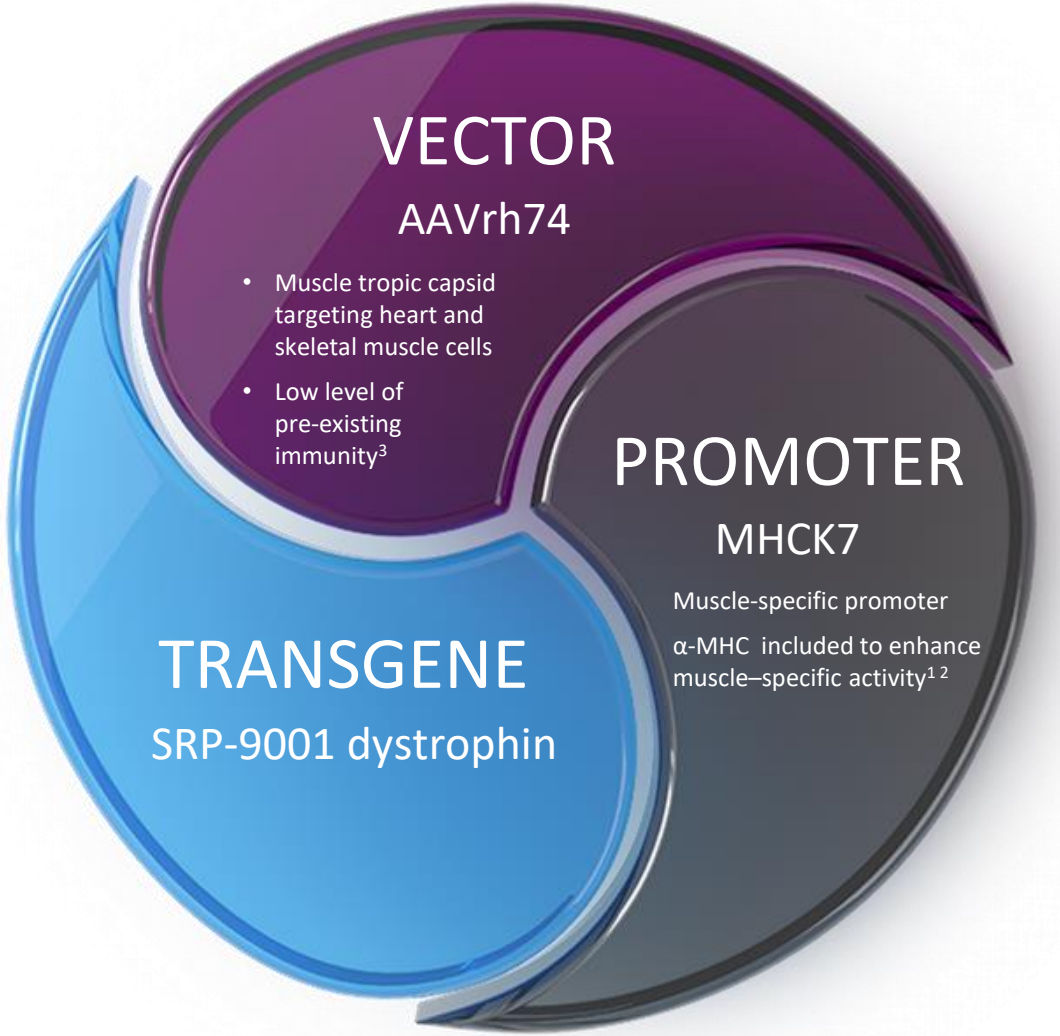


1. National Institutes of Health. Genetics Home Reference. Duchenne and Becker muscular dystrophy. https://ghr.nlm.nih.gov/condition/duchenne-and-becker-muscular-dystrophy. Accessed Jan 2020.

2. McDonald CM, Abresch RT, Duong T, et al. Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study. Lancet. 2018;3(391):451-461.

3. Passamano L, Taglia A, et al. Improvement of survival in Duchenne Muscular Dystrophy: retrospective analysis of 835 patients. Acta Myologica. 2012;31(1): 121-125.

SRP-9001 (delandistrogene moxeparvovec) Construct



1. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. ASGCT 2019.
2. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. AIM 2019.
3. Nelson DM, Ervasti JM, et al. Variable rescue of microtubule and physiological phenotypes in mdx muscle expressing different miniaturized dystrophins. Human Molecular Genetics, 2018, Vol. 27, No. 12: 2090-2100.

SRP-9001 Clinical Development Program

Sarepta has dosed the largest number of patients in Duchenne compared to any other gene therapy

ENDEAVOR

EMBARK

STUDY 101
4 patients
Ages 4-7, ambulatory
Open-Label
NCT03375164

- *Safety, proof-of-concept*
- *Enrollment completed*
- *One-year results published in JAMA Neurology*
- *Positive 2-year, 3-year and 4-year functional data*

STUDY 102
41 patients
Ages 4-7, ambulatory
Placebo-Controlled
NCT03769116

- *Safety, function*
- *Enrollment completed*
- *5-year, 3-part study*
- *Part 1 (48 weeks DBPC) and Part 2 (48 weeks blinded cross-over) completed*
- *3-year follow-up ongoing*

STUDY 103
40 patients
Ages 3+, ambulatory and non-ambulatory
Open-Label
NCT04626674

- *Expression and safety*
- *Enrollment completed for 4 cohorts*
- *Genetic mutation inclusion criteria varies by cohort*

STUDY 301
120 patients
Ages 4-7, ambulatory
Double-Blind, Placebo-Controlled
NCT05096221

- *Pivotal Phase 3 study*
- *Primary endpoint: NSAA*
- *Excludes mutations 1 to 17, 45*
- *Enrollment ongoing*

Exons 18-58

Exons 1-79

Exons 18-44, 46-79

CLINICAL SUPPLY

COMMERCIALLY REPRESENTATIVE MATERIAL

Pre-Specified External Control Analyses were Performed per FDA Guidance to Contextualize Imbalanced and Uncontrolled Data-Sets

Conservative two-step analysis to minimize confounding

1. Use ***propensity weighting*** to generate a highly matched external control group from a larger contemporary natural history and RCT data-base
2. Use a ***linear regression*** model to correct for any remaining imbalances in baseline covariates

The external control comparator was comprised of data from the following studies:

- CINRG Duchenne Natural History Study (DNHS; NCT00468832)[1,2]
- Finding the Optimum Regimen for Duchenne Muscular Dystrophy (FOR-DMD; NCT01603407)[†3]
- Lilly Study (H6D-MC-LVJJ; NCT01865084)[†4,5]

Inclusion criteria

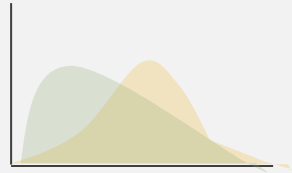
- **Age matched at baseline**
- On a stable dose or dose equivalent of oral corticosteroids for ≥12 weeks before baseline (patients on 10-day-on/10-day-off regimen will be excluded)
- **NSAA score ≥13 and ≤30 at baseline***
- **Time to Rise: ≤10.4 seconds at baseline***
- **10MWR: ≤9.1 seconds at baseline***

Analysis method

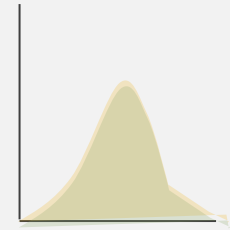
- Propensity score weighting based on:
  - Age
  - NSAA
  - Time to Rise results
  - 10MWR results

Prognostic factors in Duchenne that are known to impact function

Theoretical example



Before propensity weighting, the ranges are the same but with unequal distribution



After propensity weighting, the two populations completely overlap

†RCT data sources – reduce concerns regarding placebo effect

*Based on observed baseline values in Study 102.

10MWR, 10-meter Walk/Run; CINRG, Cooperative International Neuromuscular Research Group; DMD, Duchenne muscular dystrophy; NSAA, North Star Ambulatory Assessment.

1. Spurney C, et al. Muscle Nerve. 2014; 50:250–256; 2. ClinicalTrials.gov. NCT00468832 (Accessed March 2022); 3. ClinicalTrials.gov. NCT01603407 (Accessed March 2022); 4. Victor RG, et al. Neurology. 2017; 89:1811–1820; 5. ClinicalTrials.gov. NCT01865084 (Accessed March 2022).

Study SRP-9001-103 (ENDEAVOR) Cohort 1 (n=20)

1-year functional results

SRP-9001 is an investigational therapy and has not been reviewed or approved by any regulatory authority.

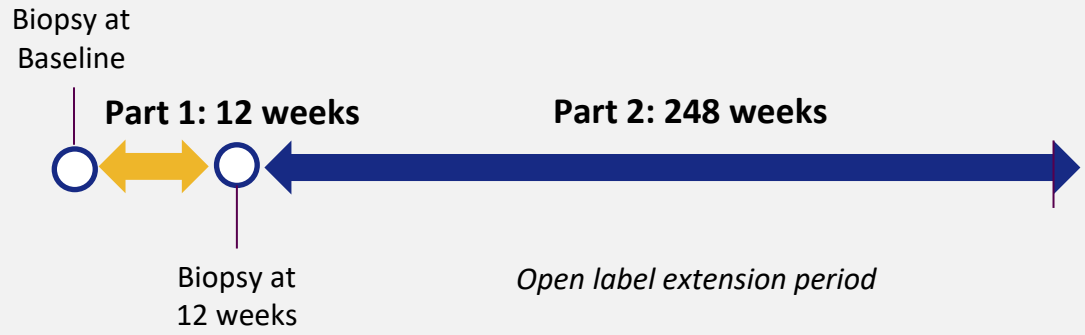
Study Design: SRP-9001-103*

STUDY 103

ENDEAVOR

- **Design:** Ongoing, multi-center open-label clinical trial
- **Objectives:** Evaluate the safety and expression of a single dose of SRP-9001 commercially representative material
- **Participants:** 40 boys with Duchenne, expanded cohorts to include older ambulant and non-ambulant individuals, and younger participants
- **Dose:** Weight-based dosing, $1.33x10^{14}$ (linear standard qPCR method)
- **Inclusion criteria:**
  - Genetic mutation inclusion criteria varies by cohort
  - Negative for AAVrh74 antibodies
- **Primary endpoint:**
  - Micro-dystrophin protein expression from Baseline to Week 12, as measured by western blot

Single IV Infusion, SRP-9001 (n=40)



	n
Cohort 1: 4-<8 years, ambulatory	20
Cohort 2: 8-17 years, ambulatory	7
Cohort 3: no age limit, non-ambulatory	6
Cohort 4: ≥3 to <4years, ambulatory	7

*ClinicalTrials.gov Identifier: NCT04626674.

SRP-9001-103 (ENDEAVOR) Cohort 1: Propensity Score Weighting Balances the Baseline Variables Well

	Study SRP-9001-103 1-year Analysis Set	
Parameter (mean)	**SRP-9001**	**External Control**
	(n=20)	(n=91)
Age	**5.8**	**6.2**
NSAA Total Score	**22.1**	**21.9**
Time to Rise from the Floor	**4.2**	**4.2**
Time of 10MWR	**5.1**	**5.1**

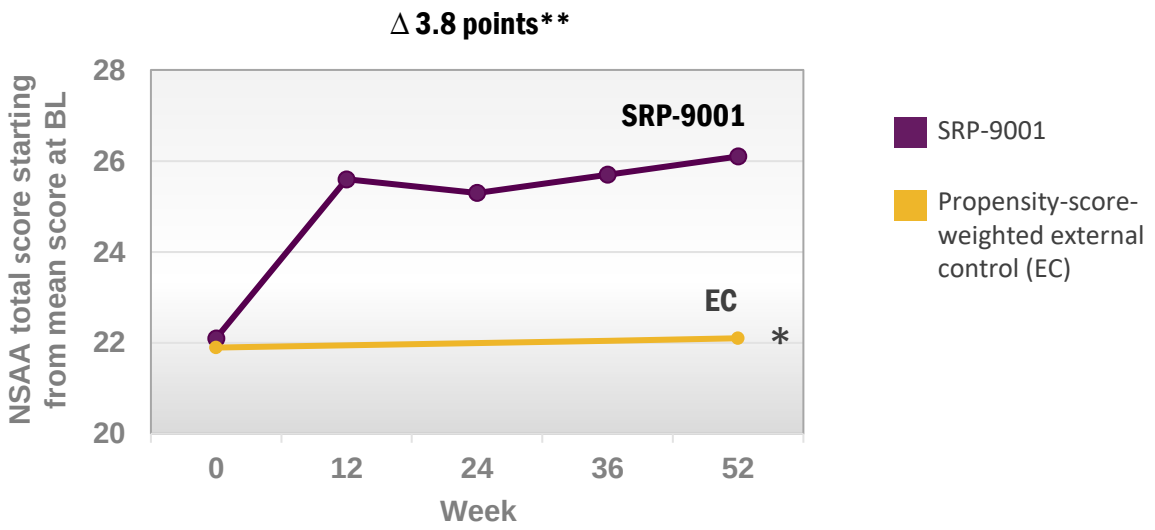
Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.

SRP-9001-103 (ENDEAVOR): 3.8-point Difference on NSAA Change from Baseline in Patients Receiving SRP-9001 Compared to External Control Group at Week 52

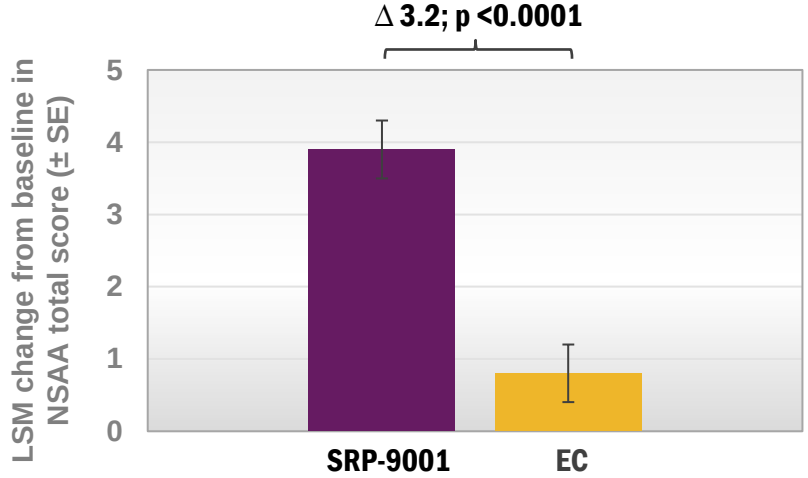
Patients treated with SRP-9001 improved 4 points from baseline

Functional Results: NSAA

NSAA total score over 1 year SRP-9001 vs External Control (unadjusted means)



NSAA change from baseline over 1 year SRP-9001 vs External Control (Least Square Means)



Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.

*Data points only available at 0 and 52 weeks for the full EC group

** NSAA change from baseline over 1 year 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.

SRP-9001-103 (ENDEAVOR) Cohort 1: Mean Improvements Observed Across Key Secondary Functional Endpoints

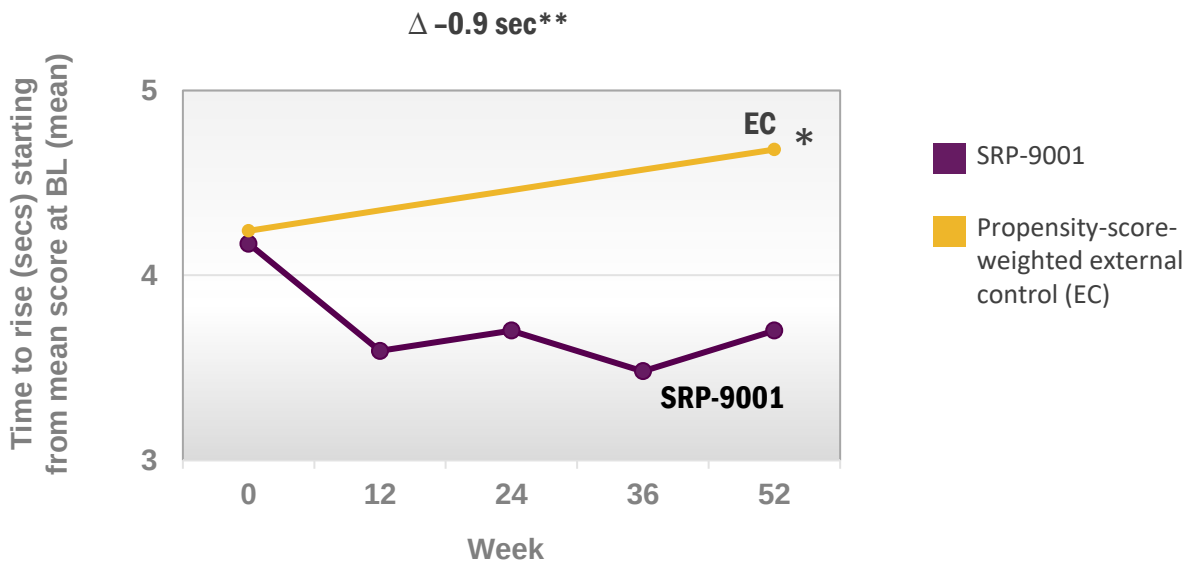
	Baseline mean (SD)	Year 1 mean (SD)	Mean change from baseline at Year 1* (SD)
Time to Rise, seconds	4.2 (1.4)	3.7 (2.1)	–0.5 (1.5)
10-meter walk/run	5.1 (0.8)	4.4 (1.0)	–0.8 (0.8)
Time to Ascend 4 Steps	3.6 (1.0)	2.8 (1.3)	–0.8 (0.9)
100-meter Walk/Run	64.1 (20.7)	52.1 (13.7)	–12.0 (18.4)

*Timed function tests are measured in seconds. Therefore, decreases in the number of seconds to complete the test following SRP-9001 treatment demonstrates improvements in motor function.

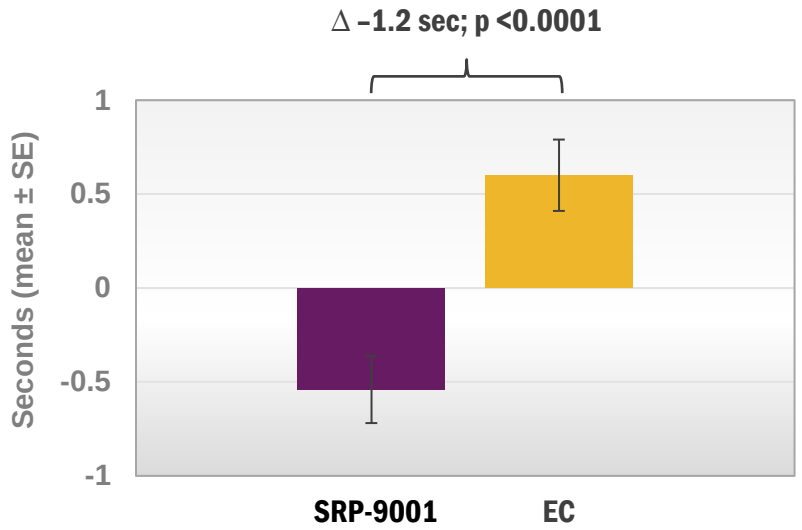
Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.

SRP-9001-103 (ENDEAVOR): Statistically Significant Result in Key Timed Function Test, Time to Rise, Compared to External Control Group at Week 52

Time to Rise (sec) over 1 year SRP-9001 vs External Control (unadjusted means)



Time to Rise over 1 year SRP-9001 vs External Control (Least Square Means change from baseline)

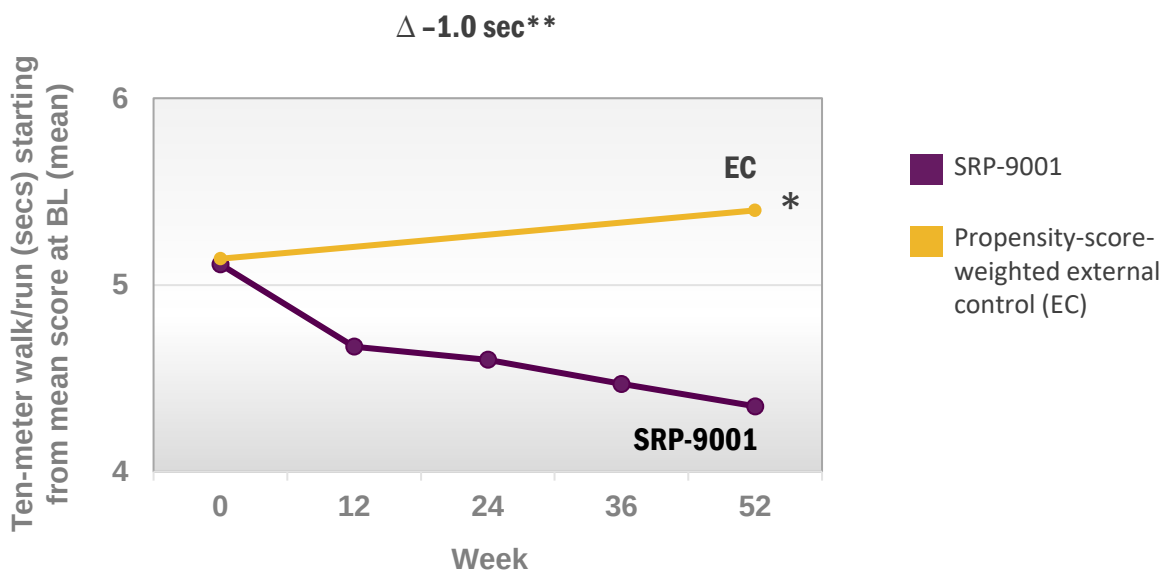


Comparisons to EC data are not available for 100-meter Walk/Run and Time to Ascend 4 steps

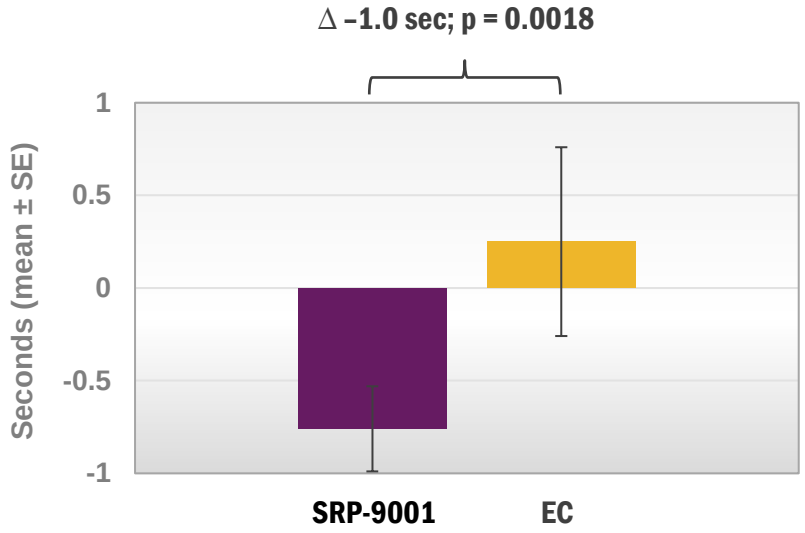
*Data points only available at 0 and 52 weeks for the full EC group
**Time to Rise change from baseline over 1 year SRP-9001 vs External Control calculated using unadjusted means
Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.
EC, external control; LSM, least square mean; SD, standard deviation; SE, standard error.

SRP-9001-103 (ENDEAVOR): Statistically Significant Result in Key Timed Function Test, Ten-Meter Walk/Run, Compared to External Control Group at Week 52

Ten-meter walk/run (sec) over 1 year SRP-9001 vs External Control (unadjusted means)



Ten-meter walk/run (sec) over 1 year SRP-9001 vs External Control (Least Square Means change from baseline)



Comparisons to EC data are not available for 100-meter Walk/Run and Time to Ascend 4 steps

*Data points only available at 0 and 52 weeks for the full EC group
**10-meter walk/run change from baseline over 1 year SRP-9001 vs External Control calculated using unadjusted means
Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.
EC, external control; LSM, least square mean; SD, standard deviation; SE, standard error.

Long-term Data

Study SRP-9001-101 (n=4)

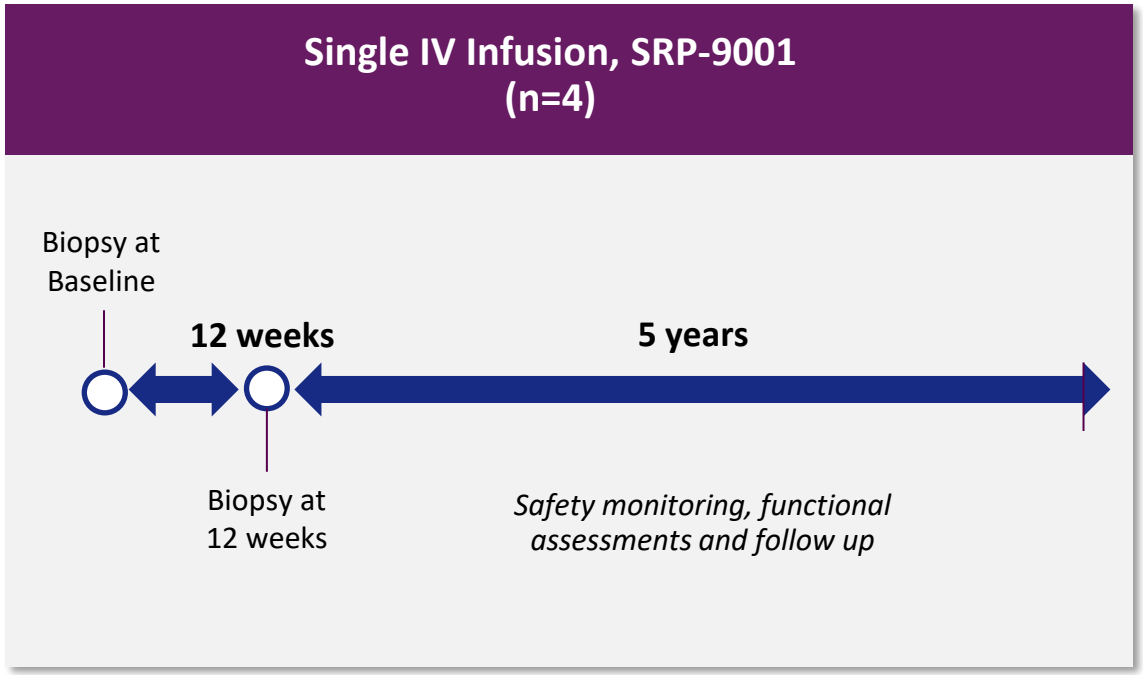
4-year follow-up data

SRP-9001 is an investigational therapy and has not been reviewed or approved by any regulatory authority.

Study Design: SRP-9001-101*

STUDY 101

- **Design:** Single center, open-label clinical trial
- **Objectives:** Evaluating the safety, tolerability and proof-of-concept of a single dose of clinical process SRP-9001
- **Participants:** 4 ambulatory boys with Duchenne, 4-7 years of age
- **Dose:** Weight-based dosing, 1.33X10^14 (linear standard qPCR method)
- **Inclusion criteria:**
  - Confirmed *DMD* mutation between exons 18-58, inclusive
  - Negative for AAVrh74 antibodies
- **Primary endpoint:**
  - Safety
- **Secondary endpoints:**
  - Change in micro-dystrophin expression pre- vs post-treatment
  - Decrease in creatine kinase (CK)
  - North Star Ambulatory Assessment (NSAA)
  - Timed function tests



*ClinicalTrials.gov Identifier: NCT03375164.

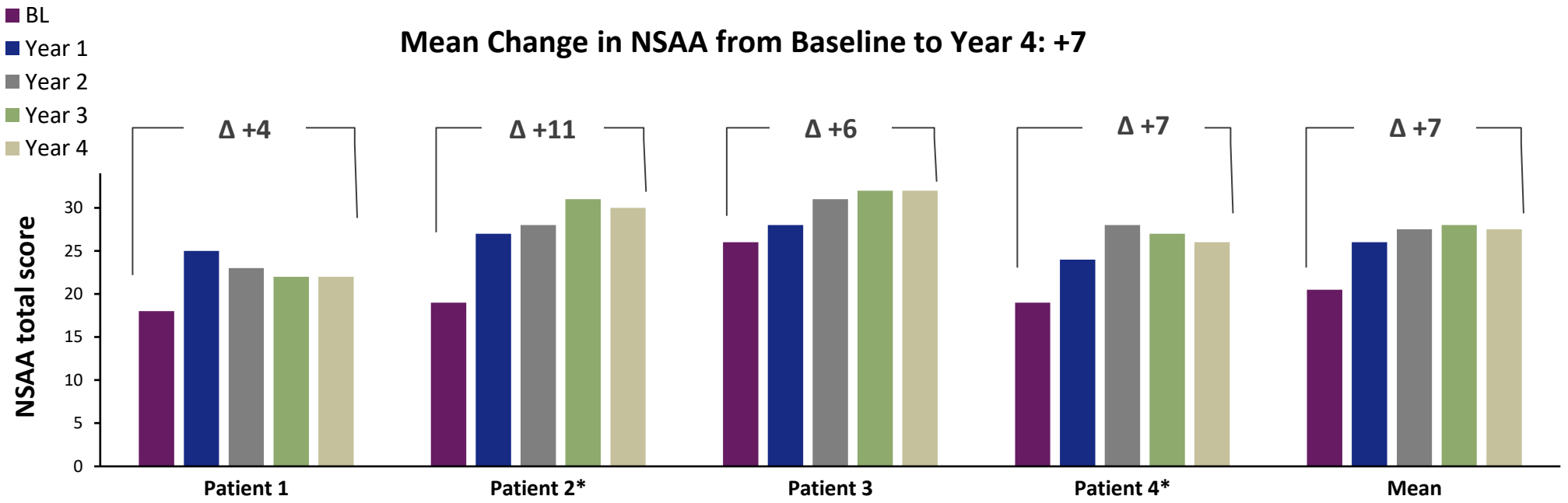
SRP-9001-101: Propensity Score Weighting Balances the Baseline Function Well

	Study SRP-9001-101 4-year Analysis Set	
Parameter (mean)	**SRP-9001**	**External Control**
	(n=4)	(n=21)
Age*	**5.1**	**6.4**
NSAA Total Score	**20.5**	**21.5**
Time to Rise from the Floor	**3.7**	**3.9**
Time of 10MWR	**4.9**	**5.0**

*Balancing for age was limited by a reduced number of suitable patients in the external control database with 4-year functional data.
Groups are well balanced for functional assessments predictive of disease progression.
Source: Data on file.

Subjects in Study SRP-9001-101 Demonstrated a Mean Increase of 7.0 Points in Total NSAA Score from Baseline to Year 4

STUDY 101



Age (years)					
At Baseline	5.7	4.8	6.0	4.0	5.1
At Year 1	6.8	5.8	7.1	5.1	6.2
At Year 2	7.7	6.8	8.0	6.3	7.2
At Year 3	8.7	7.8	9.1	7.1	8.2
At Year 4	9.7	8.8	10.1	8.1	9.2

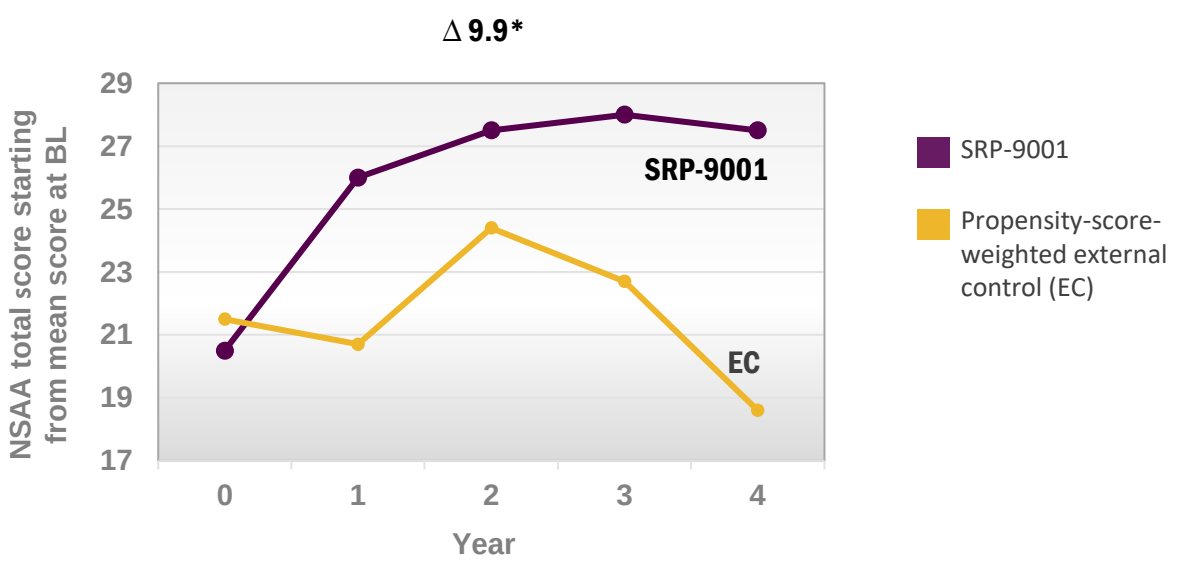
*Patient 2: 3-year NSAA value and Patient 4: 2-year NSAA value was from a remote assessment due to COVID-19 related restrictions at the site.

Mendell JR *et al*. Phase 1/2a trial of SRP-9001 in patients with Duchenne muscular dystrophy: 3-year safety and functional outcomes. Presented at the World Muscle Society Virtual Congress, 20–24 September, 2021. 2. Muntoni F, *et al*. *PLoS One.* 2019;14(9):e0221097.

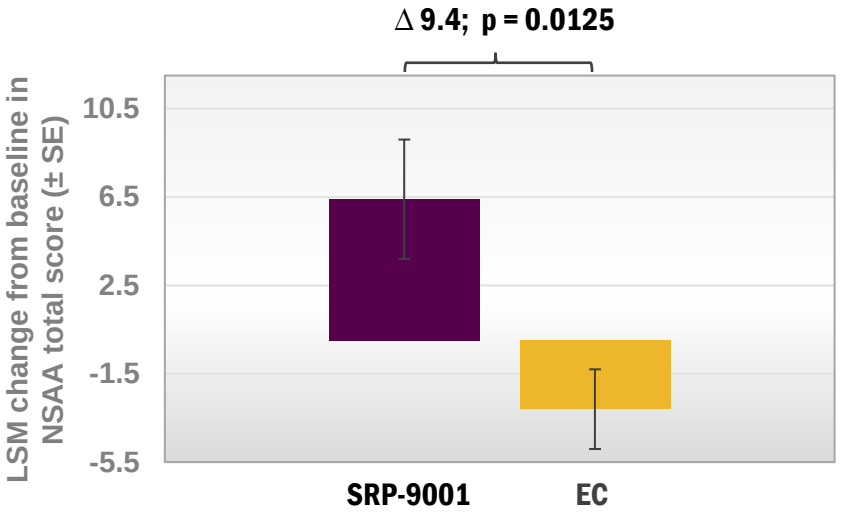
SRP-9001-101: 9.9-point Difference on NSAA Means in Patients Receiving SRP-9001 Compared to External Control Group at the End of Year 4

Functional Results: NSAA

NSAA total score over 4 years SRP-9001 vs External Control (unadjusted means)



NSAA change from baseline over 4 years SRP-9001 vs External Control (Least Square Means)



Source: Mendell, J. et al, ICNMD Conference 2022 and data on file.

*NSAA change from baseline over 4 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.

Study SRP-9001-102 Part 1 (n=20)

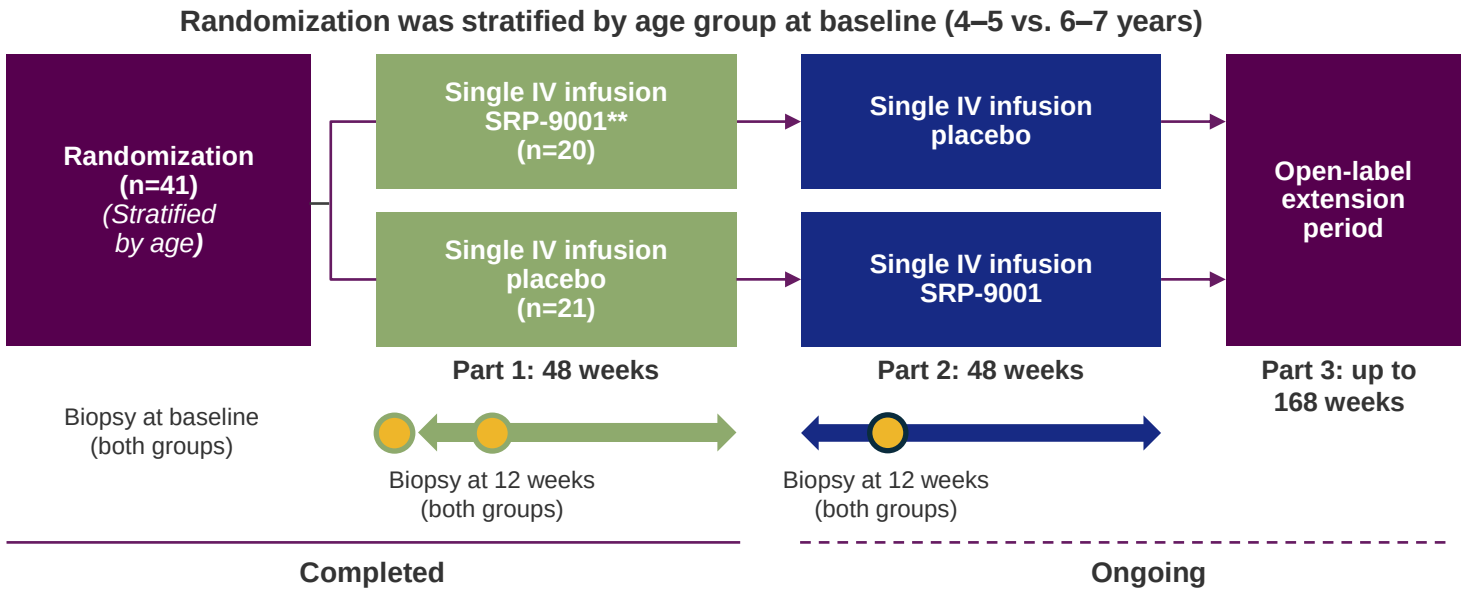
2-year functional results

SRP-9001 is an investigational therapy and has not been reviewed or approved by any regulatory authority.

Study Design: SRP-9001-102* (Parts 1 and 2)

STUDY 102

- **Design:** Ongoing, multi-center, randomized, double-blind, placebo-controlled clinical trial; remains blinded
- **Objectives:** Evaluating the safety, efficacy and tolerability of a single dose of clinical process SRP-9001 compared to placebo
- **Participants:** 41 ambulatory boys with DMD, 4-7 years of age
- **Dose:** Weight-based dosing, $1.33x10^{14}$** (linear standard qPCR method)
- **Inclusion criteria:**
  - Confirmed *DMD* mutation between exons 18-58, inclusive
  - Negative for AAVrh74 antibodies
- **Primary endpoints:**
  - Micro-dystrophin protein expression, from Baseline to Week 12, as measured by western blot
  - Change in NSAA total score from Baseline to Week 48



***All patients received the target dose as determined by the supercoiled standard qPCR method specified in the protocol at the time. Subsequent retrospective analysis using the new linear standard qPCR method indicated that 60% of the patients received a dose lower than the target dose based on the new method. All patients going forward will receive the target dose as determined by the new method. Target dose 2E14 vg/kg was estimated by supercoiled standard qPCR and is equivalent to 1.33E14 vg/kg using the linear standard qPCR method.*

*ClinicalTrials.gov Identifier: NCT03769116.

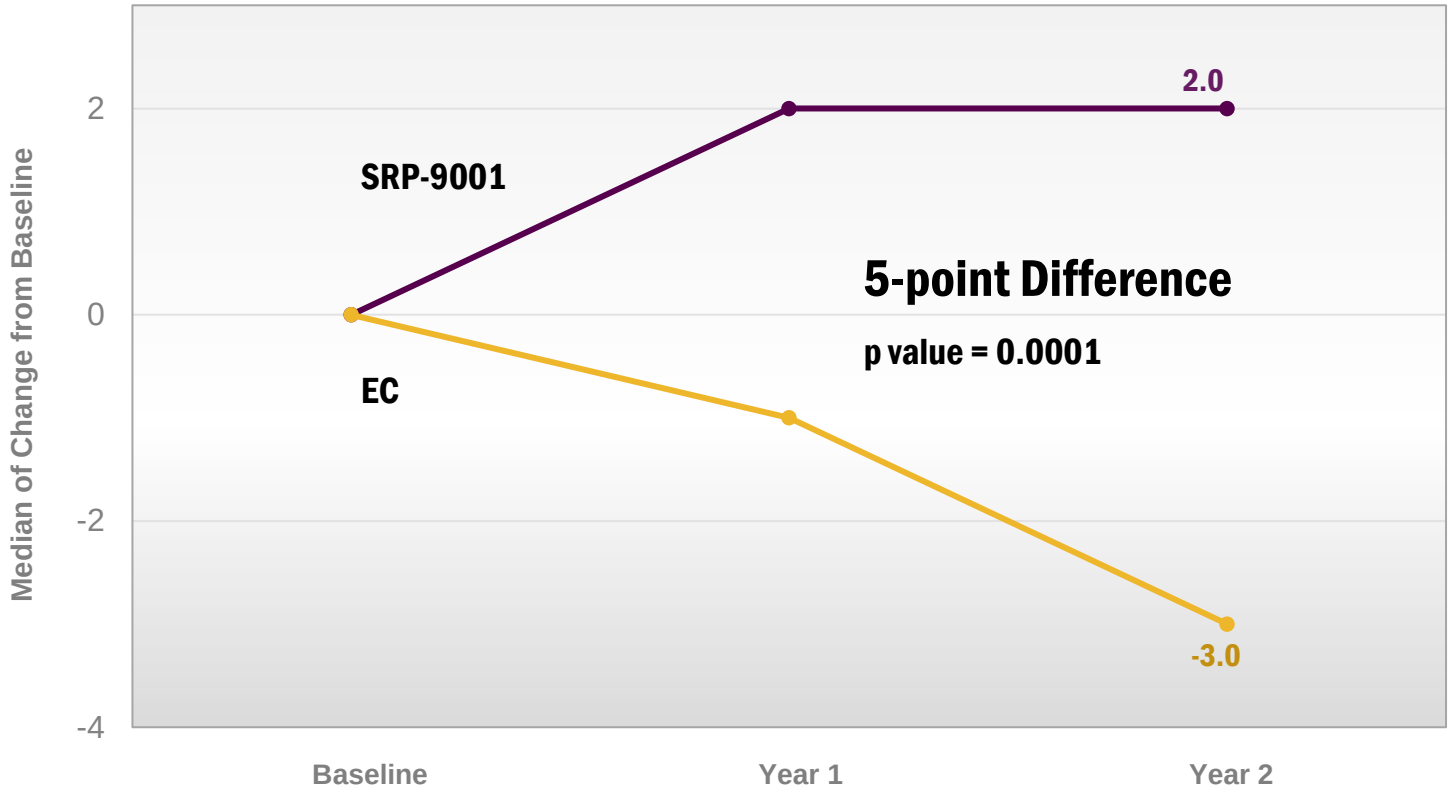
SRP-9001-102: Propensity Score Weighting Balanced the Baseline Variables Well

	Study SRP-9001-102 2-year Analysis Set	
Parameter (mean)	**SRP-9001**	**External Control**
	(n=19)	(n=51)
Age	**6.2**	**6.2**
NSAA Total Score	**19.9**	**19.7**
Time to Rise from the Floor	**5.2**	**5.2**
Time of 10MWR	**5.4**	**5.4**

Source: Data on file.

SRP-9001-102 (Part 1): 96-Week Data

Significant 5-point median NSAA difference from baseline in SRP-9001 patients compared to propensity matched external control at week 96



Change from baseline NSAA over 2 years

Parameter	SRP-9001 (n=19)	External Control (n=51)
Least Square Mean	1.6	-0.4
Median	2	-3
Min, Max	-17, 9	-11, 10

An extreme value in the SRP-9001 arm substantially negatively skews the data making post hoc comparisons of the medians the appropriate analysis (p value =0.0001).

Source: Data on file.

Integrated Efficacy Analysis from SRP-9001-101, 102 and 103

1-year functional results compared to external control (EC)

SRP-9001 is an investigational therapy and has not been reviewed or approved by any regulatory authority.

Integrated Analyses: Data from Clinical Trials of SRP-9001 in Duchenne

Functional data from patients who received the target dose were pooled from 3 studies

SRP-9001-101

An ongoing study evaluating the safety, efficacy and tolerability of a single IV dose of SRP-9001*

Study n=4

Analysis n=4

Ambulatory boys with Duchenne aged ≥4 to <8 years

Clinical process material

SRP-9001-102

An ongoing **Phase 2** study evaluating the safety, efficacy and tolerability of a single IV dose of SRP-9001†, compared with placebo

Study n=41

Analysis n=28‡

Boys with Duchenne aged ≥4 to <8 years

Clinical process material

SRP-9001-103 (ENDEAVOR)

An ongoing open-label, **Phase 1b** study to assess the expression and safety of commercially representative SRP-9001 material

Study n=40

Analysis n=20§

Boys with Duchenne

- **Cohort 1** (ambulatory, ≥4 to <8 years)
- **Cohort 2** (ambulatory, ≥8 to <18 years)
- **Cohort 3** (non-ambulatory)
- **Cohort 4** (ambulatory, ≥3 to <4 years)

Commercially representative material

*The dose of delandistrogene moxeparvovec in Study 101 was 2.0×10^{14} vg/kg determined by supercoiled qPCR method (equivalent to 1.33×10^{14} vg/kg using qPCR with linear standard). †The intended target dose in Study 102 was 1.33×10^{14} vg/kg delandistrogene moxeparvovec IV infusion compared with placebo infusion. The 1.33×10^{14} vg/kg dose in Study 102 is the same as the 2.0×10^{14} dose previously used in Study 101. The difference is due to changes in PCR quantification methods. ‡The 28 patients who received the target dose in Study 102 were analyzed. §The 20 patients in Cohort 1 were analyzed. One-year data from Cohorts 2-4 are not yet available and will be presented at the next update.
IV, intravenous; PCR, polymerase chain reaction; qPCR, quantitative PCR.

Data ICNMD 2022

Integrated Analysis: Propensity Score Weighting Balances the Baseline Variables Well

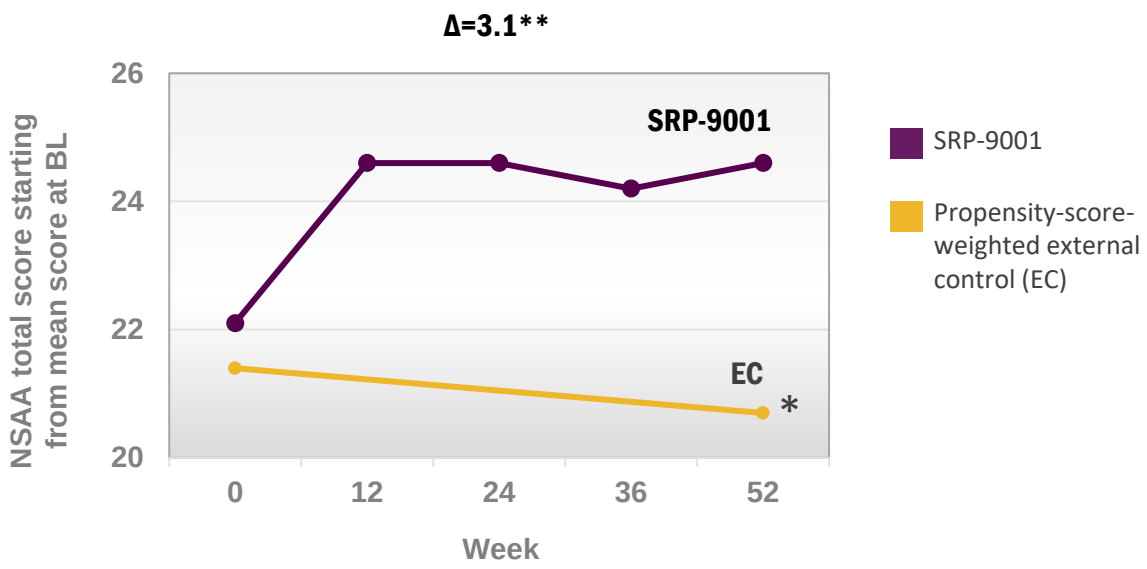
	1-year Integrated Analysis Set	
Parameter (mean)	SRP-9001	External Control
	(n=52)	(n=105)
Age	6.4	6.7
NSAA Total Score	22.1	21.4
Time to Rise from the Floor	4.5	4.5
Time of 10MWR	5.1	5.2

Source: Zaidman, C. et al, ICNMD Conference 2022.

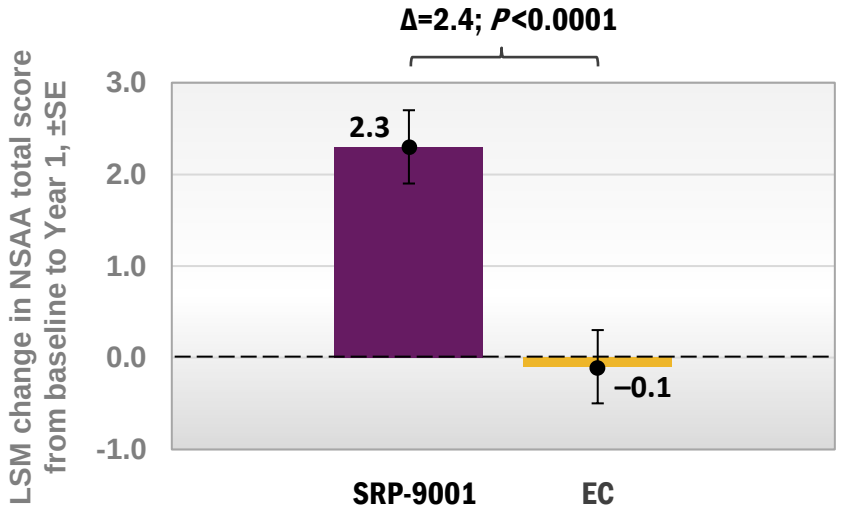
Integrated Analyses: Statistically Significant NSAA Total Score Compared to External Control Group at 52 Weeks

Functional Results: NSAA

NSAA total score over 1 year SRP-9001 vs External Control (unadjusted means)



NSAA change from baseline over 1 year SRP-9001 vs External Control (Least Square Means)



*Data points only available at 0 and 52 weeks for the full EC group.

**NSAA change from baseline over 1 year SRP-9001 vs External Control calculated using unadjusted means.

131 EC participants were used to derive the propensity scores. After the propensity scores were derived, 26 participants were removed because their propensity scores were outside the range of the treated patients. Therefore, in the comparative analysis, only 105 patients were included.

Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.

EC, external control; LSM, least-squares mean; NSAA, North Star Ambulatory Assessment; SE, standard error.

Integrated Analyses: Mean Improvements Observed Across Key Secondary Functional Endpoints

Functional Results: Timed Function Tests

	Baseline mean (SD)		Year 1 mean (SD)		Unadjusted Mean change from baseline at Year 1 (SD)			LSM change from baseline at Year 1 (SE)			
	Integrated (n=52)	EC (n=105)	Integrated (n=52)	EC (n=101–103)	Integrated (n=52)	EC (n=101–103)	Difference between Integrated Analysis and EC**	Integrated (n=52)	EC (n=101–103)	Difference between Integrated Analysis and EC**	P-value
Time to Rise, seconds	4.5 (1.8)	4.5 (1.2)	4.1 (2.1)	5.6 (2.7)*	-0.4 (1.1)	1.2 (2.12)*	-1.5	-0.5 (0.2)	1.0 (0.2)	-1.6	<0.0001
10-meter walk/run, seconds	5.1 (1.1)	5.2 (0.7)	4.9 (1.6)	5.7 (1.9)†	-0.2 (1.0)	0.6 (1.7)†	-0.8	- 0.2 (0.2)	0.5 (0.2)	-0.7	0.0164

*N=101; †N=103;
EC, external control.
**Raw means July 2022
Source: Zaidman, C. et al, ICNMD Conference 2022 and data on file.

SRP-9001 Dystrophin Expression, Transduction, and Localization from the Clinical Development Program

Measure	Timepoint	Study 101 (Early Development Process) (n=4)	Study 102 Part 1 & 2 Target Dose [a] (Early Development Process) (n=29)	Study 103 (Intended Commercial Process) (n=20)
Mean age (years) at time of biopsy	W12	5.4	7.4	6.1
Vector Genome Copy Number [b]	Mean change from Baseline to W12 (range)	**3.3**	**2.9**	**3.4**
		(1.3 - 8.1)	(0.3 - 7.3)	(0.7-9.8)
SRP-9001 Dystrophin Expression (western blot, % of normal expression)	Mean change from Baseline to W12 (range)	**70.5**	**38.6**	**54.2**
		(13.5 - 182.6)	(-1.1 - 114.7)	(4.8-153.9)
IF Fiber Intensity (% control)	Mean change from Baseline to W12 (range)	**93.6 [c]**	**61.6**	**66.5**
		(58.8 - 157.8)	(-7.7 - 138.1)	(-9.6 - 263.6)
PDPF, %	Mean change from Baseline to W12	**81.2 [c]**	**64.1**	**48.3**
		(73.5 - 96.2)	(-7.3 - 96.1)	(1.1 - 84.4)

IF = immunofluorescent; PDPF = percent dystrophin positive fibers.
Data extraction date: 9001-101: 15 June 2021; 9001-102: 12 May 2021; 9001-103: 09 February 2022
[a] Target Dose = 1.33 x 10^{14} vg/kg by ddPCR
[b] qPCR was used to analyze vector genome copies in Study SRP-9001-101; ddPCR was used for Studies SRP-9001-102 and -103.
[c] IF and PDPF values in Study SRP-9001-101 were calculated using different methods than those used in SRP-9001-102 and -103.

Safety Analyses

SRP-9001 is an investigational therapy and has not been reviewed or approved by any regulatory authority.

Most Common Treatment-emergent Adverse Events (TEAEs)

Collective safety data from all patients in SRP-9001-101 and 102, and all cohorts of ENDEAVOR (SRP-9001-103), n=84

Safety Results: TEAEs Occurring in at Least 25% of All Participants

	Target dose (n=72)	All (n=84)
Vomiting, n (%)	45 (62.5)	52 (61.9)
Decreased appetite, n (%)	35 (48.6)	40 (47.6)
Nausea, n (%)	31 (43.1)	34 (40.5)
Upper respiratory tract infection, n (%)	23 (31.9)	34 (40.5)
Pain in extremity, n (%)	16 (22.2)	24 (28.6)
Abdominal pain upper, n (%)	18 (25.0)	23 (27.4)
Irritability, n (%)	17 (23.6)	23 (27.4)
Procedural pain, n (%)	14 (19.4)	22 (26.2)

Source: Zaidman, C. et al, ICNMD Conference 2022.
TEAE, treatment emergent adverse event.

Treatment-related Serious Adverse Events

- Seven patients (8.3%) experienced treatment-related SAEs
- Treatment-related SAEs included:
  - Vomiting (2)
  - Liver injury (1)
  - Increased transaminases (2)
  - Rhabdomyolysis (2)
  - Immune-mediated myositis (1)*
  - Myocarditis (1)
    - 11-year-old boy initially admitted to treat nausea and vomiting
    - Raised troponin was noted incidentally during his hospitalization, with no symptoms/signs of systolic dysfunction
    - Function was preserved on ECHO and cardiac MRI, but MRI findings were consistent with myocarditis superimposed on DMD cardiomyopathy
    - The patient received 3 days of IV methyl-prednisolone
    - Post event: additional chronic cardiac medications added, cardiac MRI (1 month) showed normal function and partial resolution of myocarditic changes, and ECHO (4 months) showed normal systolic function

Source: Zaidman, C. et al, ICNMD Conference 2022.
*This event has been disclosed previously.
ECHO, echocardiogram; IV, intravenous; MRI, magnetic resonance imaging; SAE, serious adverse event; SUSAR, suspected unexpected serious adverse reaction.

Summary & Next Steps

Clinical Data Show SRP-9001's Positive Effect on Duchenne Disease Progression

New data from SRP-9001-101, 102, 103 and integrated analysis (SRP-9001-101, 102, 103) demonstrate consistently meaningful results

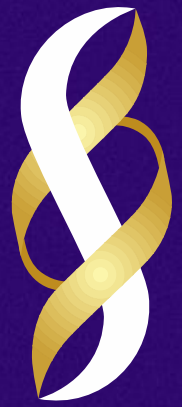
- **Clinical Impact**
  - **SRP-9001-103 (ENDEAVOR):** Data generated from commercially representative material at target dose further reinforce our confidence in SRP-9001-301 (EMBARK)
  - **Integrated Efficacy Analysis (SRP-9001-101, 102 and 103):** Robust data set shows consistency across all 3 studies when compared to external control

- **Durability:** 2- and 4-year functional data from Studies 102 and 101 suggest SRP-9001 alters the trajectory of the disease, stabilizing function which is sustained over time

- **Consistent and Manageable Safety Profile**
  - Broad patient experience (including patients over 80kg) has, to date, observed a safety profile that is consistent and manageable (only using single drug steroid regimen)
  - No clinically relevant complement activation was observed

- **Path Forward:** Regulatory discussions ongoing; update when complete

Q&A



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