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

So neither will we.

SAREPTA THERAPEUTICS

TRYNITY
Living with limb-girdle muscular dystrophy

Winter 2024

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 our opportunities in the rare disease space; potential solutions and market opportunities with our RNA technologies, gene therapy and gene editing; the potential benefits of our manufacturing processes; the potential benefits of our collaborations and partnerships; the potential benefits of our technologies and scientific approaches, including the potential of RNA-targeted medicine to increase or decrease production of a protein involved in a disease; the potential benefits of PMO and PPMO, including PPMO’s potential to greatly increase cell penetration, lead to more efficient dosing and greater benefit for patients, deliver to unique muscle types and treat Duchenne; the potential benefits of ELEVIDYS; the potential benefits of SRP-5051, including the safety profile and SRP-5051’s potential to treat patients with Duchenne amenable to exon 51 skipping and potentially lead to more efficient dosing and greater clinical benefit for patients; the potential benefits of SRP-9003; the potential benefits of MyoAAV, including the potential to generate greater efficacy (i.e., restoration of muscle function) at lower therapeutic doses; the potential benefits of MHCK7, AAVrh74, SR2, SR3 and β-sarcoglycan; the potential of our LGMD portfolio to generate a steady stream of gene therapy candidates in additional subtypes representing more than 70% of all known LGMDs; the potential of gene therapy’s applicability across disease; the estimated number of patients suffering from Duchenne and LGMD; our understanding that the 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 for ELEVIDYS; and expected milestones and plans, including the review goal date of June 21, 2024 for the ELEVIDYS supplement, requesting a meeting to discuss results of MOMENTUM with FDA, expected timing Q3 2024, and plans regarding future clinical trials for LGMD and the development of future PPMOs for other exons in Duchenne and other indications.

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: if there are significant delays in obtaining or we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all, which could impair our ability to generate sufficient revenue and have a successful business; our data for our different programs, including PPMO and gene therapy-based product candidates, may not be sufficient for obtaining regulatory approval; we may not be able to comply with all FDA post-approval commitments and requirements with respect to our products in a timely manner or at all; our product candidates, including those with strategic partners, may not result in viable treatments suitable for commercialization due to a variety of reasons, including 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; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; 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; if the actual number of patients living with Duchenne and LGMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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, including research and development and the potential commercialization of our gene therapy product candidates; we may not be able to successfully scale up manufacturing of our product candidates in sufficient quality and quantity or within sufficient timelines; current reimbursement models may not accommodate the unique factors of our gene therapy product candidates; 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, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) and in its other SEC filings.

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.

~10,000 rare diseases

80%+ are single gene mutations

And only a few hundred rare diseases currently have treatments

https://ncats.nih.gov/trnd/projects/gene-therapy Accessed June 2023.

Sarepta Therapeutics, Inc. (Nasdaq: SRPT), a fully integrated biopharmaceutical company, discovers, develops and commercializes medicines that treat ***rare, genetic-based diseases.***

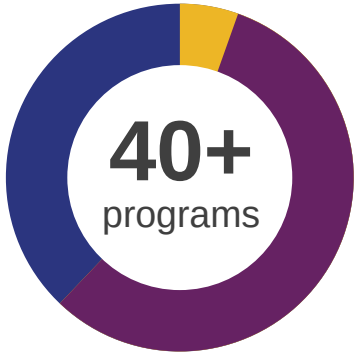
DARREN
Living with Duchenne muscular dystrophy

A differentiated corporate profile

3 **proprietary technology platforms**

- RNA
- Gene Therapy
- Gene Editing

Deep, advancing pipeline spanning several disease areas



Late-stage programs
in Duchenne and LGMD2E/R4

Substantial revenue-generating business

Driven by **4** on-market therapies

With revenues over **$1B**

ELEVIDYS

(delandistrogene moxeparvovec-rokl)

First gene therapy to treat Duchenne; granted accelerated approval by FDA (06/22/23); priority review granted, with a review goal date of June 21, 2024 (02/16/24)

Distinct partnering strategy to build a sustainable pipeline and reach more patients

Three proprietary technology platforms – RNA, gene therapy and gene editing

RNA

Exon skipping bypasses an error in the RNA, allowing the body to skip the mutation to make a functional version of the missing protein

Proprietary Exon Skipping Technology
Sarepta's exon skipping therapies act on the RNA, allowing the body to skip the mutation to make a functional version of the missing protein

GENE THERAPY

Gene therapy adds a functional copy (a transgene) of a missing or malfunctioning gene, so that the body can make a functional version of the missing or dysfunctional protein

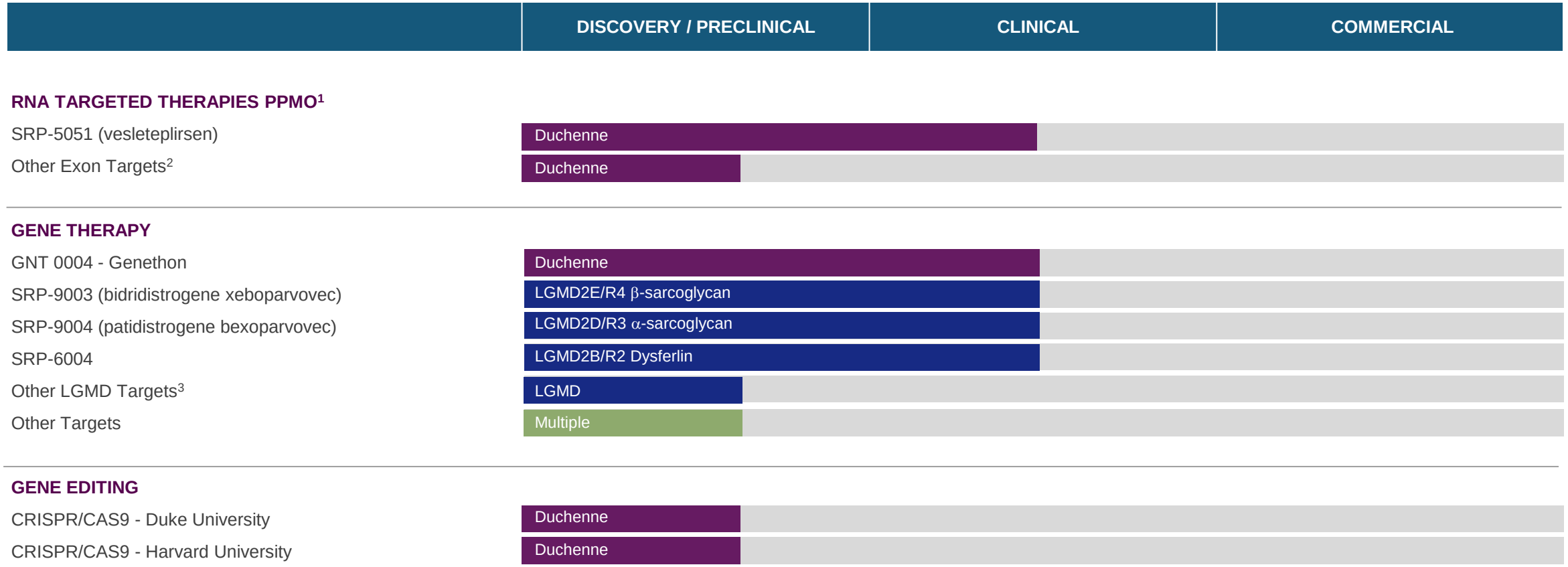
Distinct Constructs
Sarepta's gene therapy constructs are tailored to specific disease states; components are selected based on their ability to target specific tissues and cells with the goal of optimizing expression of the select protein in those tissues

GENE EDITING

One kind of gene editing restores protein expression by removing—or excising—exons that contain a genetic mutation, allowing the body to produce a functional version of the missing or dysfunctional protein

A New Approach
Differentiated scientific approach that utilizes a proprietary dual cut strategy for predictable and accurate editing

Deep, advancing pipeline

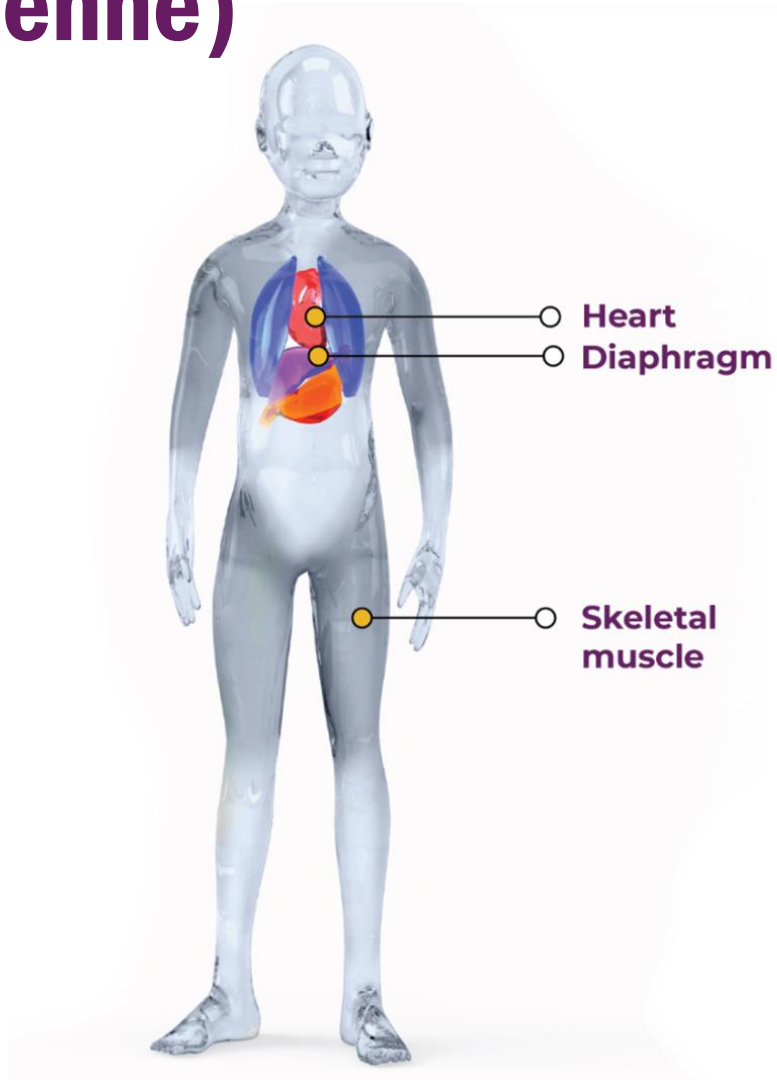


1. Peptide phosphorodiamidate morpholino oligomers
2. Other exon targets in development: 44, 45, 50, 52, and 53
3. Other LGMD targets in development: SRP-9005 (LGMD2C/R5 γ-sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and SRP-9010 (LGMD2A/R1)

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.

Disease progression in Duchenne[1-3]

5 TO 7 YEARS

- Motor delay
- Enlarged calves
- Toe walking
- Standing from supine, climbing stairs more difficult

8 TO 11 YEARS

- Increasing loss of walking ability
- Part-time wheelchair use

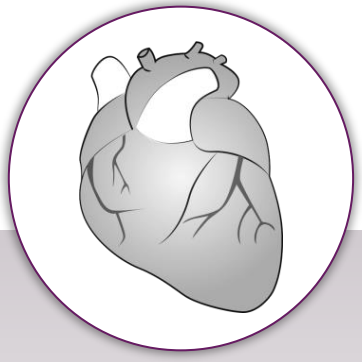
EARLY TEENS

- Loss of ambulation
- Full-time wheelchair use
- Increasing loss of upper limb function

TEENS

- Increasing respiratory impairment
- Ventilatory support often required
- Unable to perform activities of daily living

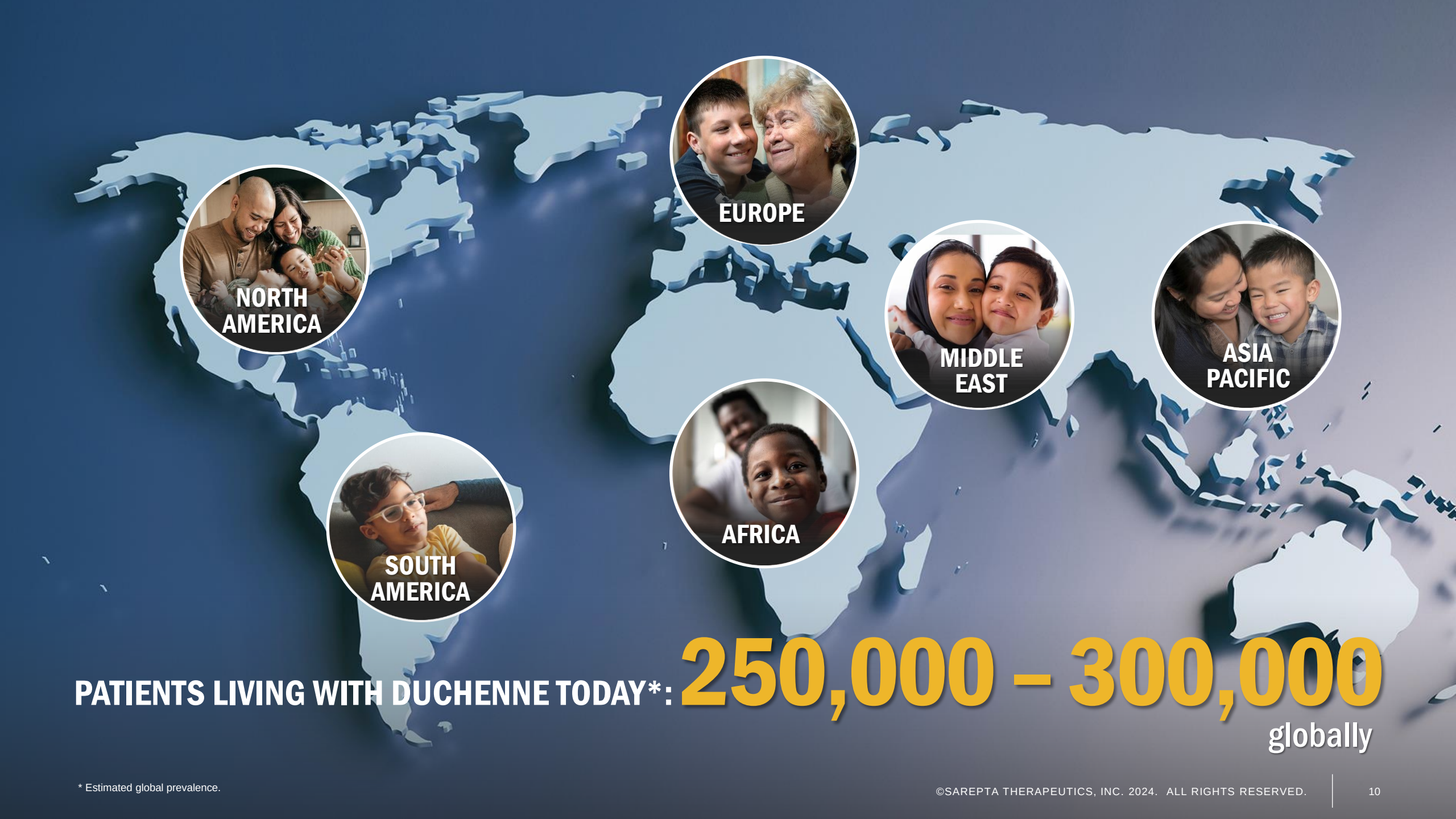
TEENS TO TWENTIES



- Increasing cardiac dysfunction
- Heart failure
- Death

EARLY AMBULATORY → LATE AMBULATORY → EARLY NON-AMBULATORY → LATE NON-AMBULATORY

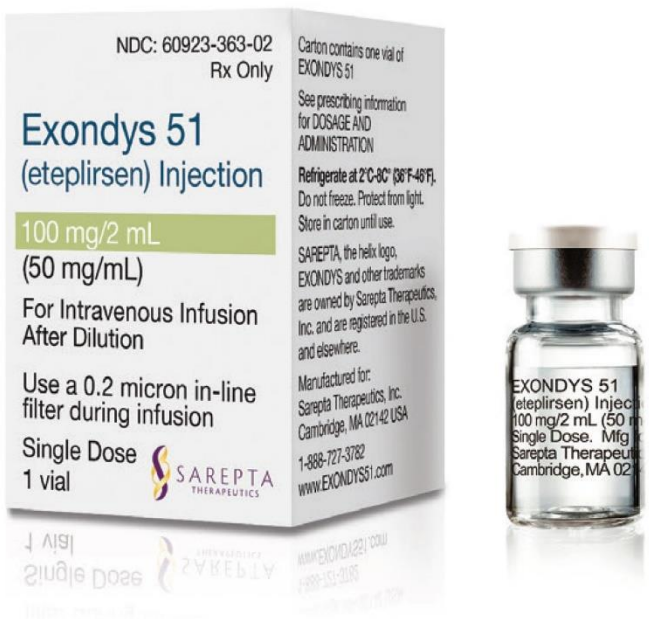
1. Bushby K, Finkel R, Birnkrant DJ, et al. *Lancet Neurol.* 2010;9:77-93.
2. Emery AEH. *Lancet*. 2002;359:687-695.
3. Landfeldt E, Lindgren P, Bell CF, et al. *Neurology*. 2014;83(6):529-536.



PATIENTS LIVING WITH DUCHENNE TODAY*: 250,000 – 300,000 globally

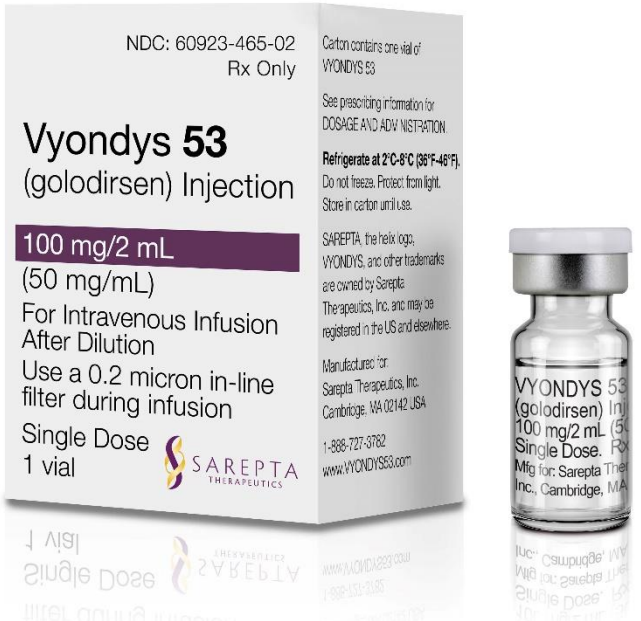
* Estimated global prevalence.

Serving approximately 30% of the Duchenne community with RNA-based therapies



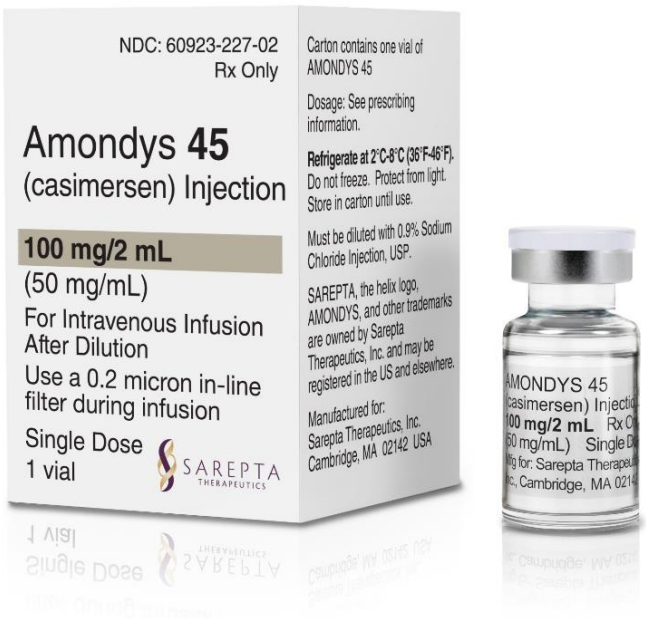
September 2016

Approved to treat patients with a confirmed genetic mutation that is amenable to exon 51 skipping (13% of Duchenne population)*



December 2019

Approved to treat patients with a confirmed genetic mutation that is amenable to exon 53 skipping (8% of Duchenne population)*

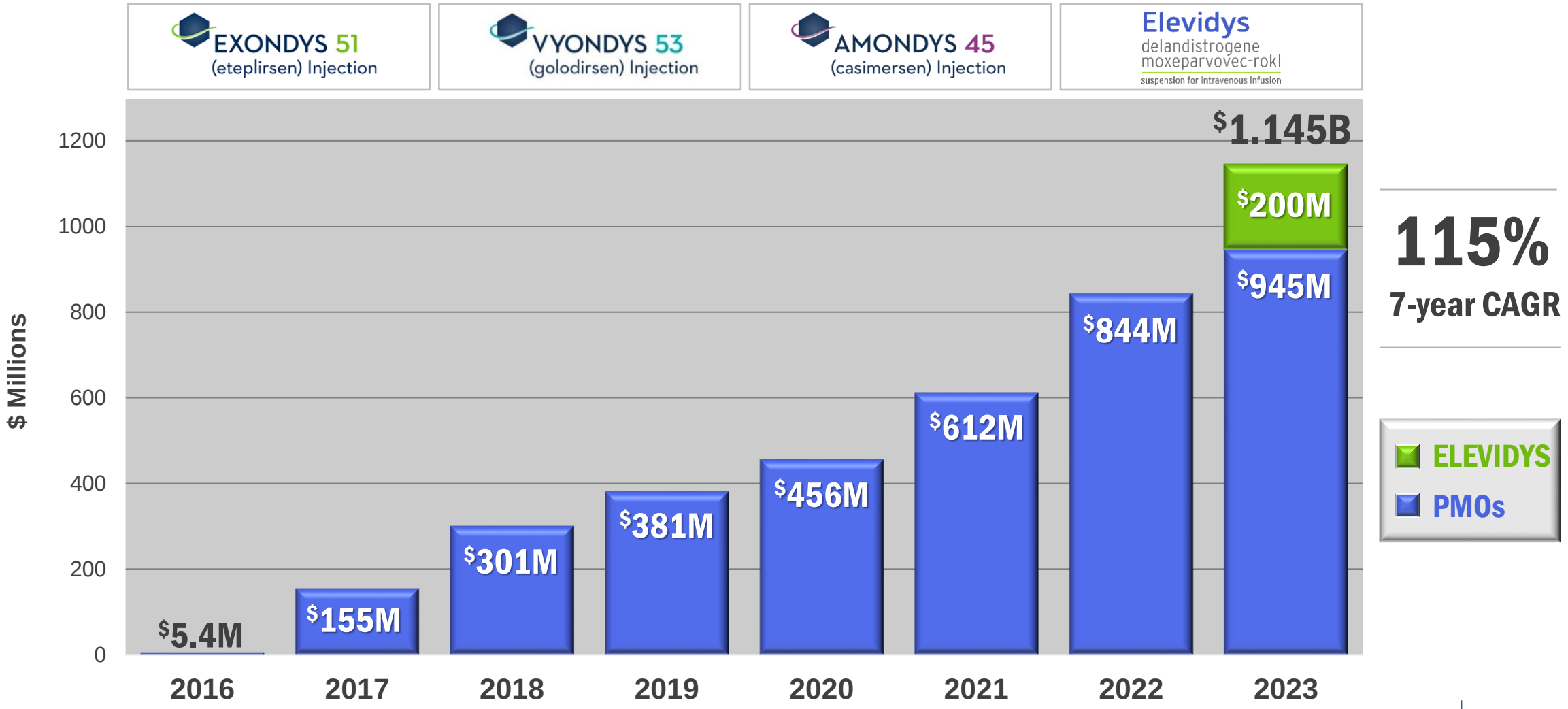


February 2021

Approved to treat patients with a confirmed genetic mutation that is amenable to exon 45 skipping (8% of Duchenne population)*

*Candidate received accelerated approval in the U.S., confirmatory studies required.

Robust total net product revenue for Duchenne franchise (PMOs and gene therapy)



ELEVIDYS (delandistrogene moxeparvovec-rokl): Gene therapy for Duchenne

ELEVIDYS* clinical development program

Accelerated Approval granted by FDA on June 22, 2023, for Duchenne patients aged 4 through 5 years; on February 16, 2024, priority review granted, with a review goal date of June 21, 2024

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

Exons 18-58

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

ENDEAVOR

STUDY 103
58 patients

Ages 3+, ambulatory and non-ambulatory

Open-Label

NCT04626674

- Expression and safety
- Enrollment completed for 4 cohorts; enrollment underway for 5th cohort
- Genetic mutation inclusion criteria varies by cohort

Exons 1-79

EMBARK

STUDY 301
125 patients

Ages 4-7, ambulatory

Double-Blind, Placebo-Controlled

NCT05096221

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

Exons 18-44, 46-79

ENVISION

STUDY 303
~148 patients

Ambulatory and non-ambulatory

Double-Blind, Placebo-Controlled

NCT05881408

- Phase 3 study
- 128 weeks, 2-part study
- Primary outcome (Part 1): Change from baseline at Week 72 in the total score of PUL**
- Study underway

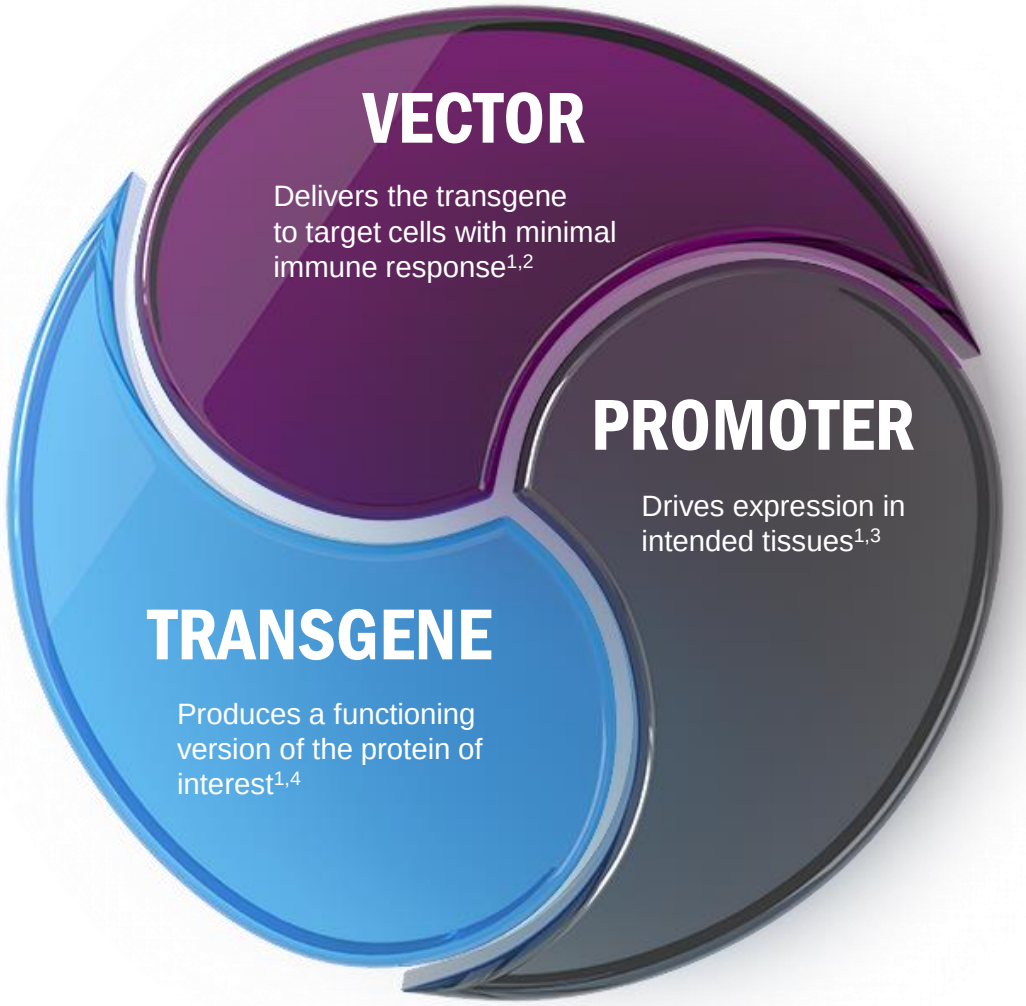
Exons 18-79

CLINICAL SUPPLY INTENDED COMMERCIAL PROCESS MATERIAL

**PUL= performance of upper limb

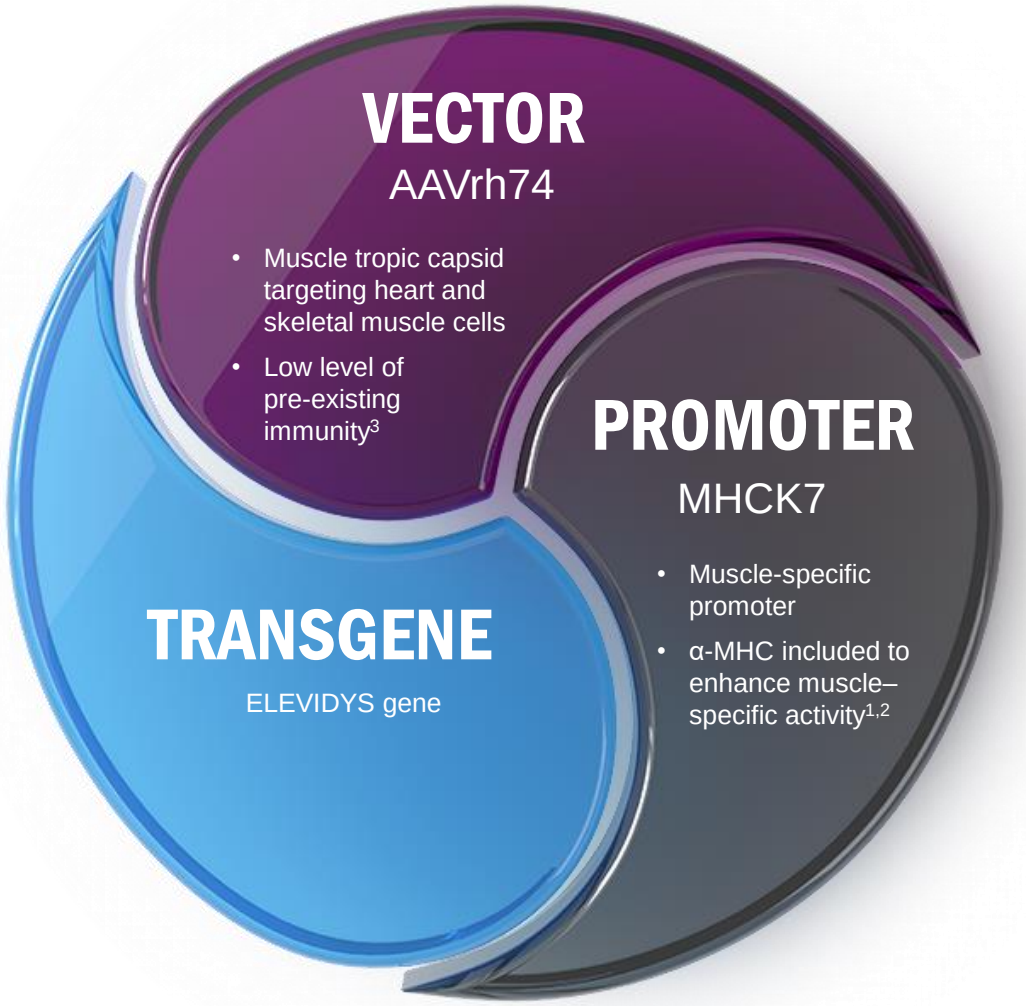
*ELEVIDYS is contraindicated in patients with any deletion in exons 8 and/or 9. Roche has commercial rights outside of the United States.

Critical components of any gene therapy



1. Asher DR, et al. Clinical development on the frontier: gene therapy for Duchenne muscular dystrophy. *Expert Opin Biol Ther*. 2020;20(3):263-274.
2. US National Library of Medicine. Help Me Understand Genetics: Gene Therapy. https://ghr.nlm.nih.gov/primer/therapy/genetherapy. Accessed Nov. 22, 2021.
3. Zheng C, Baum BJ. Evaluation of promoters for use in tissue-specific gene delivery. *Methods Mol Biol*. 2008;434:205-219.
4. Chandler RJ, Venditti CP. Gene Therapy for Metabolic Diseases.*Transl Sci Rare Dis*. 2016;1(1):73-89.

A differentiated construct in ELEVIDYS



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.

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

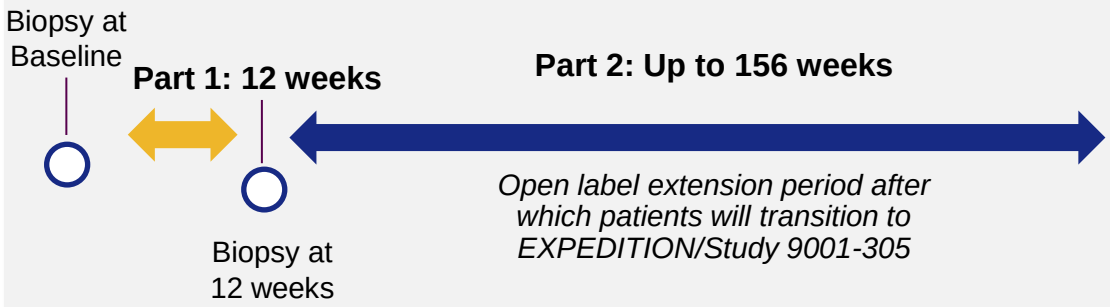
1-year functional results

Study design: SRP-9001-103

ENDEAVOR

- **Design:** Ongoing, multi-center open-label clinical trial
- **Objectives:** Evaluate the safety and expression of a single dose of SRP-9001 intended commercial process material
- **Participants:** 58 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:**
  - SRP-9001 expression from baseline to Week 12, as measured by western blot

Single IV Infusion, SRP-9001 (n=58)



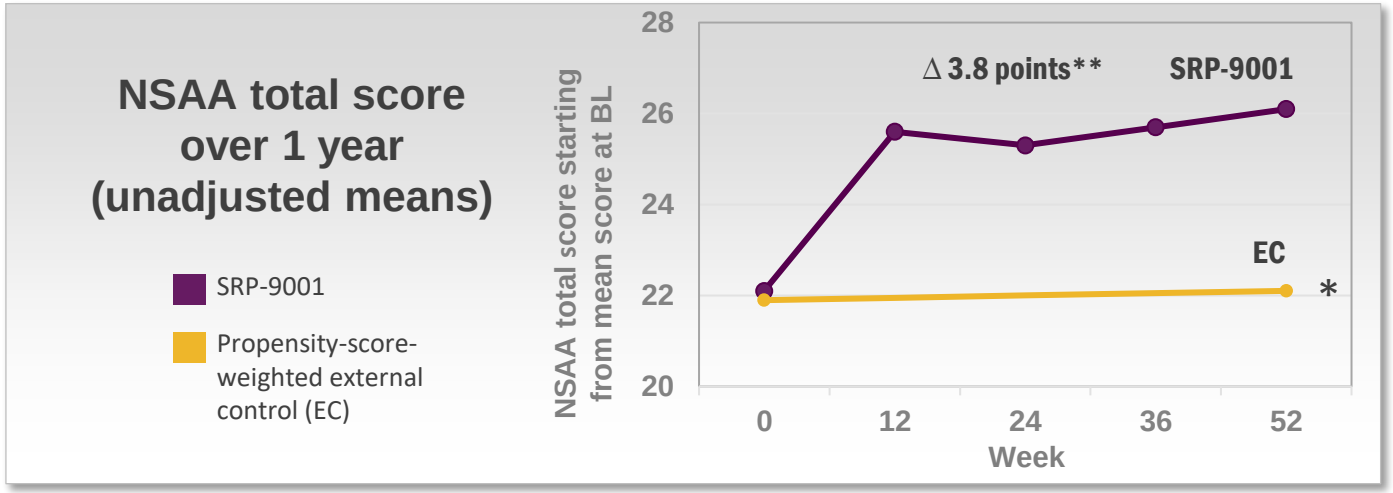
	n
Cohort 1*: ≥4 to <8 years, ambulatory	20
Cohort 2: ≥8 to <18 years, ambulatory	7
Cohort 3: no age limit, non-ambulatory	6
Cohort 4: ≥3 to <4years, ambulatory	7
Cohort 5**: ambulatory and non-ambulatory	8
Cohort 6: ≥2 to <3 years, ambulatory	6
Cohort 7: non-ambulatory	6

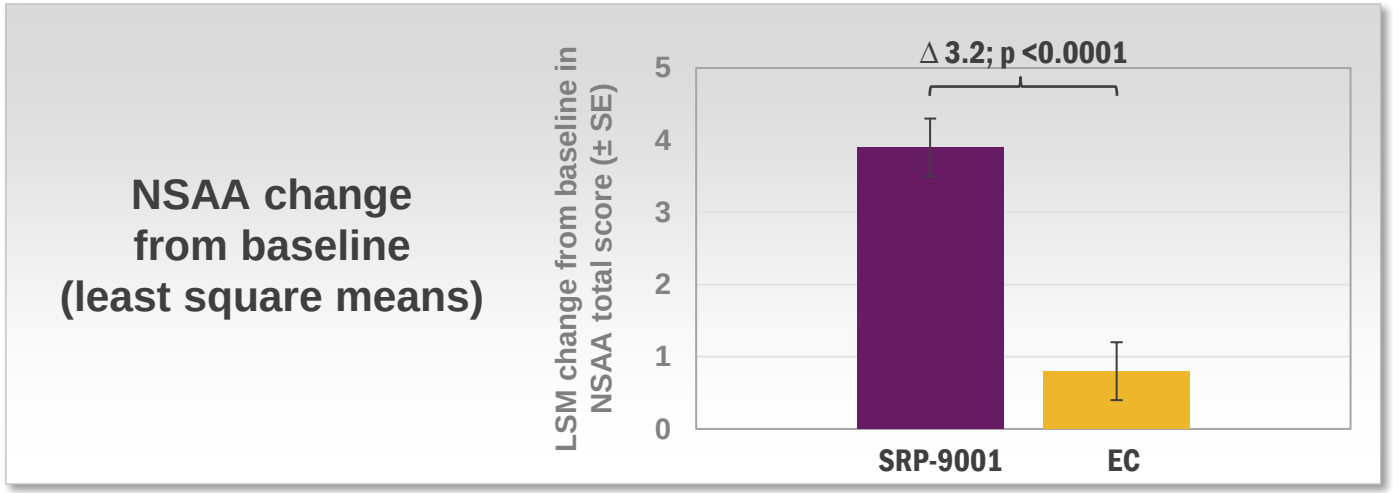
*Cohort 1 patients are included in the 1-year functional results as noted on slides 24 and 25.
**Cohort 5 is to expand beyond mutation exclusions in most SRP-9001 studies conducted to date; 8 patients (6 ambulatory plus 2 non-ambulatory).
Cohort 5a: ambulatory and ≥4 to <9 years of age at the time of Screening.
Cohort 5b: Non-ambulatory per protocol specified criteria at the time of Screening.

3.8-point difference on NSAA in patients receiving SRP-9001 compared to external control at 1 year

Study SRP-9001-103 1-year Analysis Set		
Parameter (mean)	**SRP-9001 (n=20)**	**External Control (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.





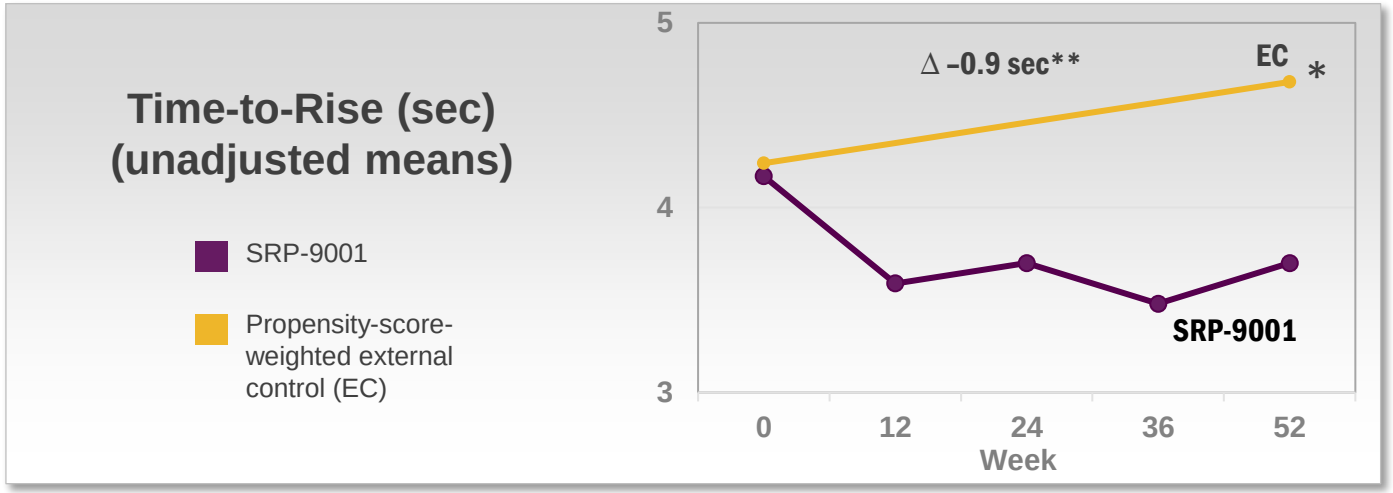
*Data points only available at 0 and 52 weeks for the full EC group.
**NSAA change from baseline over 1 year SRP-9001 vs External Control calculated using unadjusted means.
BL, baseline; EC, external control; LSM, least square mean; NSAA, North Star Ambulatory Assessment; SD, standard deviation; SE, standard error.

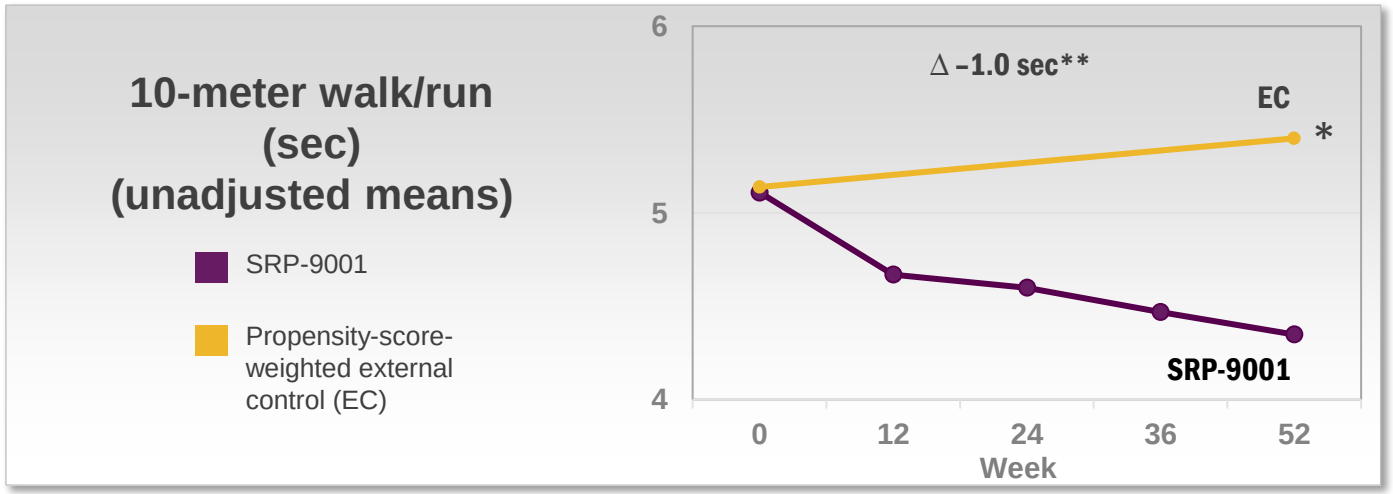
Statistically significant difference in time-to-rise and 10-meter walk/run compared to external control group at 1 year

Study SRP-9001-103 1-year Analysis Set			
Secondary Endpoints	**Baseline** Mean (SD)	**Year 1** Mean (SD)	**Δ From Baseline[1]** Mean (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)

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

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





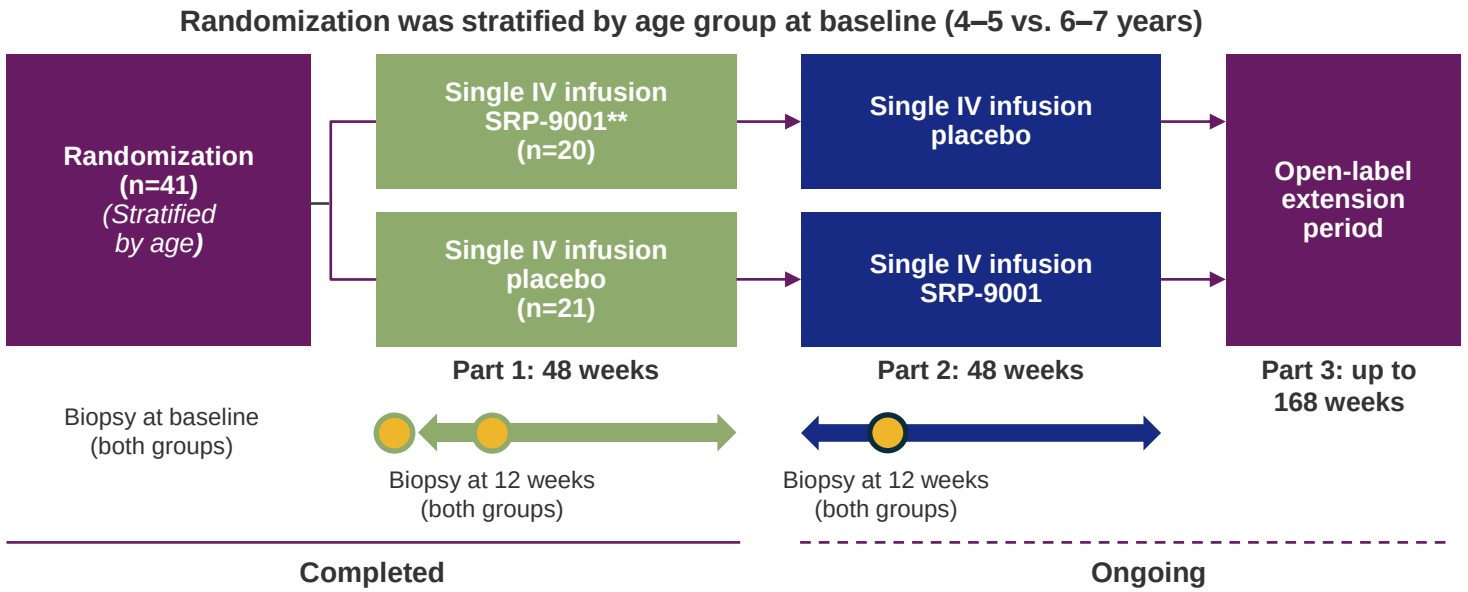
1. Timed function tests are measured in seconds. Therefore, decreases in the number of seconds to complete the test following SRP-9001 treatment demonstrates improvements in motor function.
*Data points only available at 0 and 52 weeks for the full EC group.
**Time to Rise change from baseline over 1 year SRP-9001 vs External Control calculated using unadjusted means. 10-meter walk/run change from baseline over 1 year SRP-9001 vs External Control calculated using unadjusted means.

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

2-year functional results

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

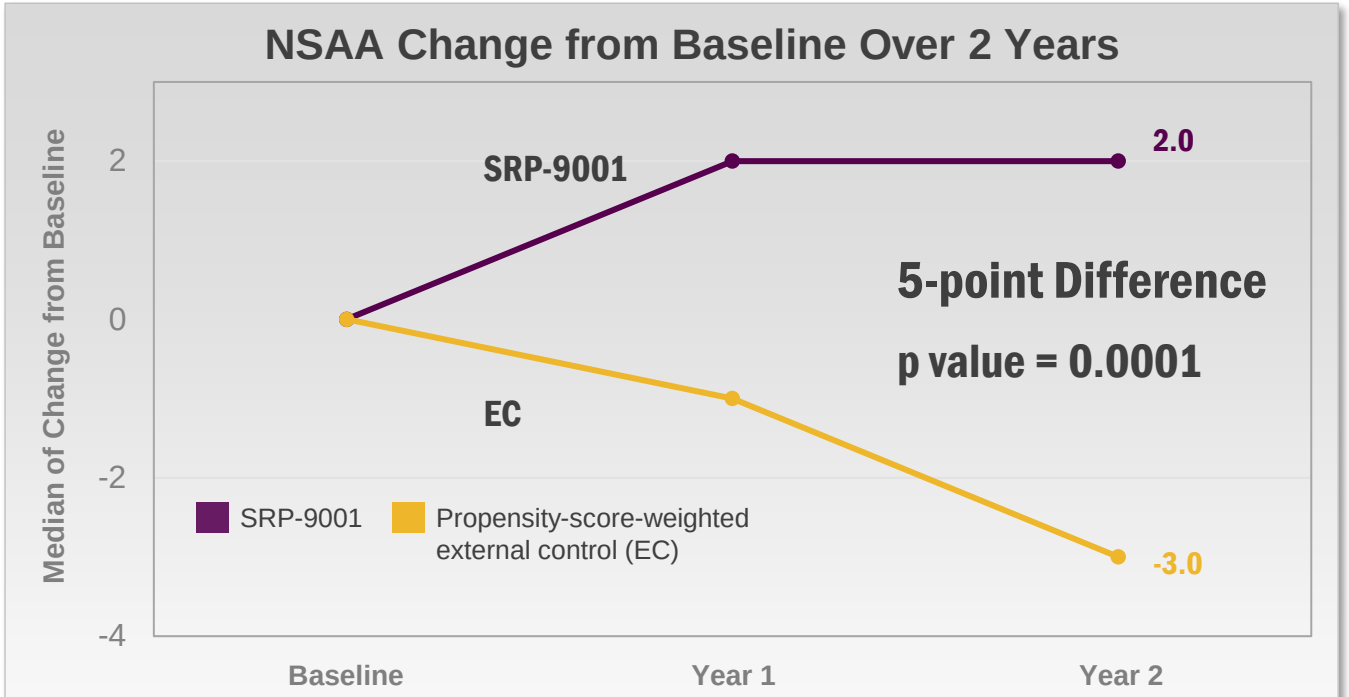
- **Design:** Ongoing, multi-center, randomized, double-blind, placebo-controlled clinical trial; remains blinded
- **Objectives:** Evaluating the safety, efficacy and tolerability of a single dose of clinical process SRP-9001 compared to placebo
- **Participants:** 41 ambulatory boys with Duchenne, 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 endpoints:**
  - SRP-9001 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.*

Year 2: Significant 5-point median NSAA difference from baseline in SRP-9001 patients compared to propensity-matched external control

Study SRP-9001-102 2-year Analysis Set		
Parameter (mean)	**SRP-9001 (n=19)**	**External Control (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



Parameter	SRP-9001	External Control
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).

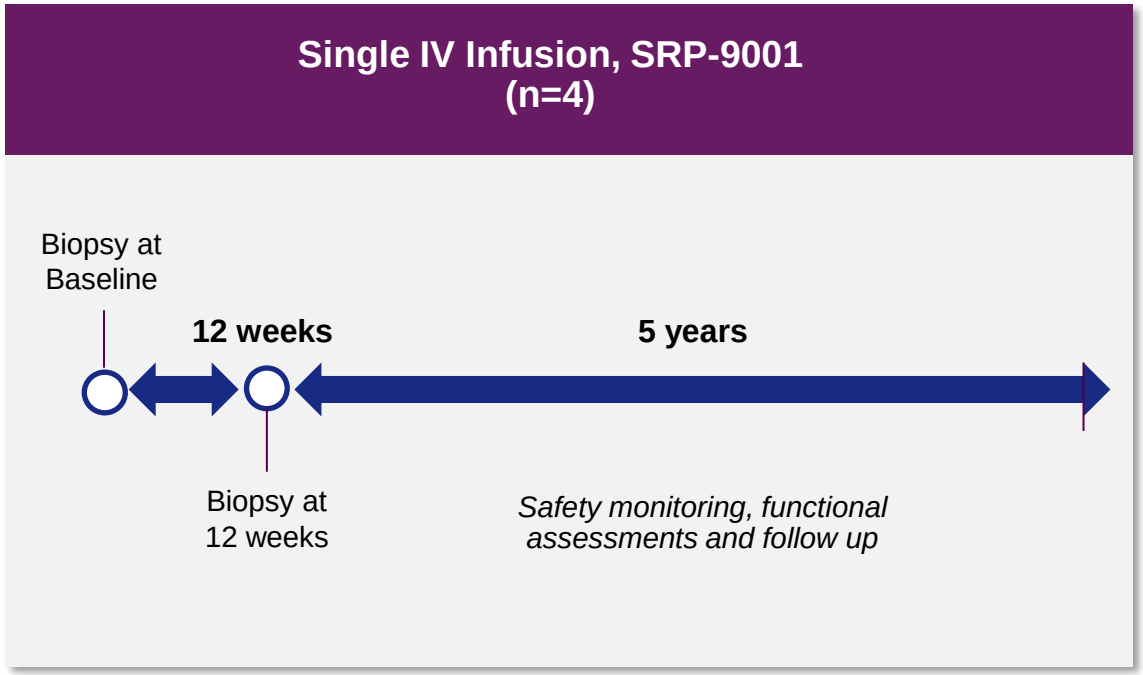
Source: Data on file.

Study SRP-9001-101 (n=4)

4-year follow-up data

Study design: SRP-9001-101

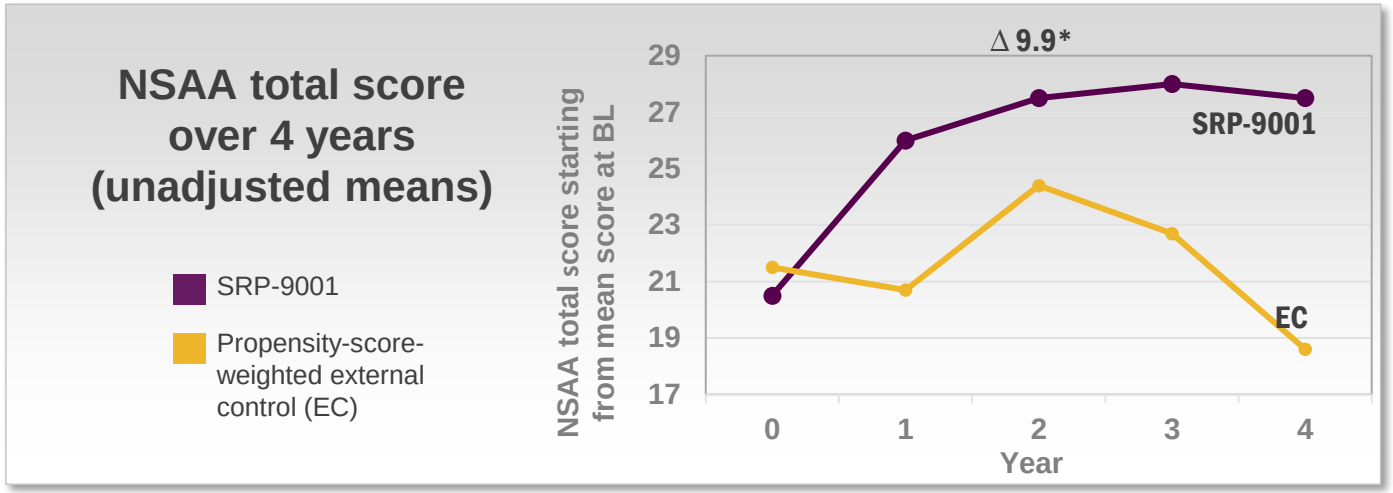
- **Design:** Single center, open-label clinical trial
- **Objectives:** Evaluating the safety, tolerability and proof-of-concept of a single dose of clinical process SRP-9001
- **Participants:** 4 ambulatory boys with Duchenne, 4-7 years of age
- **Dose:** Weight-based dosing, $2.0x10^{14}$ vg/kg by supercoiled qPCR method (equivalent to $1.33x10^{14}$ vg/kg by linear qPCR standard)
- **Inclusion criteria:**
  - Confirmed *DMD* mutation between exons 18-58, inclusive
  - Negative for AAVrh74 antibodies
- **Primary endpoint:**
  - Safety
- **Secondary endpoints:**
  - Change in SRP-9001 expression pre- vs post-treatment
  - Decrease in creatine kinase (CK)
  - North Star Ambulatory Assessment (NSAA)
  - Timed function tests

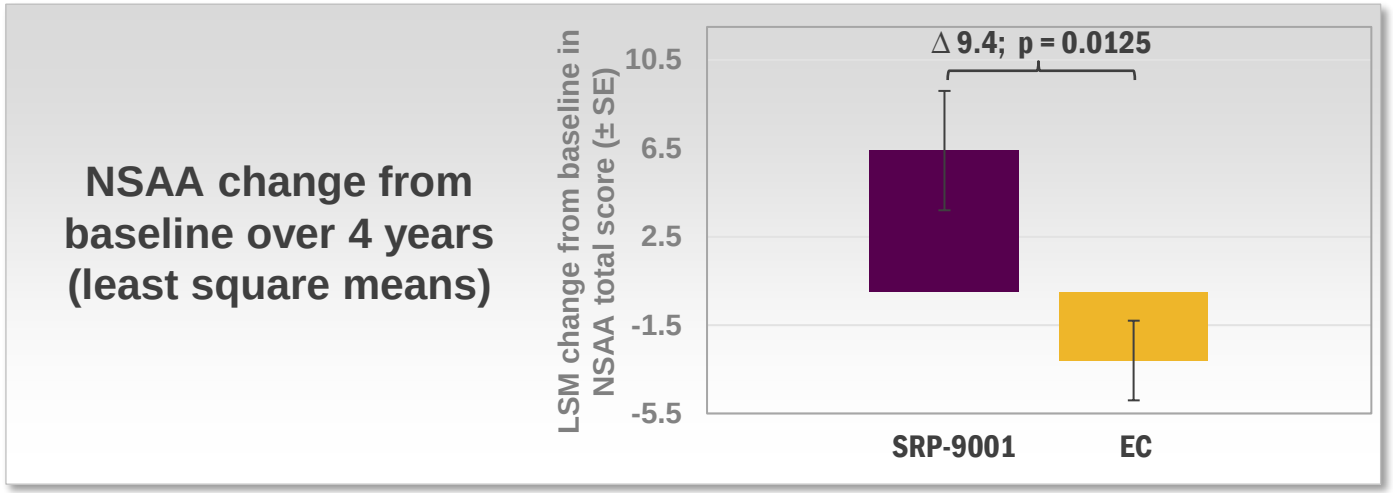


Year 4: 9.9-point difference on mean NSAA in patients receiving SRP-9001 compared to external control group

Study SRP-9001-101 4-year Analysis Set		
Parameter (mean)	**SRP-9001 (n=4)**	**External Control (n=21)**
Age[1]	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

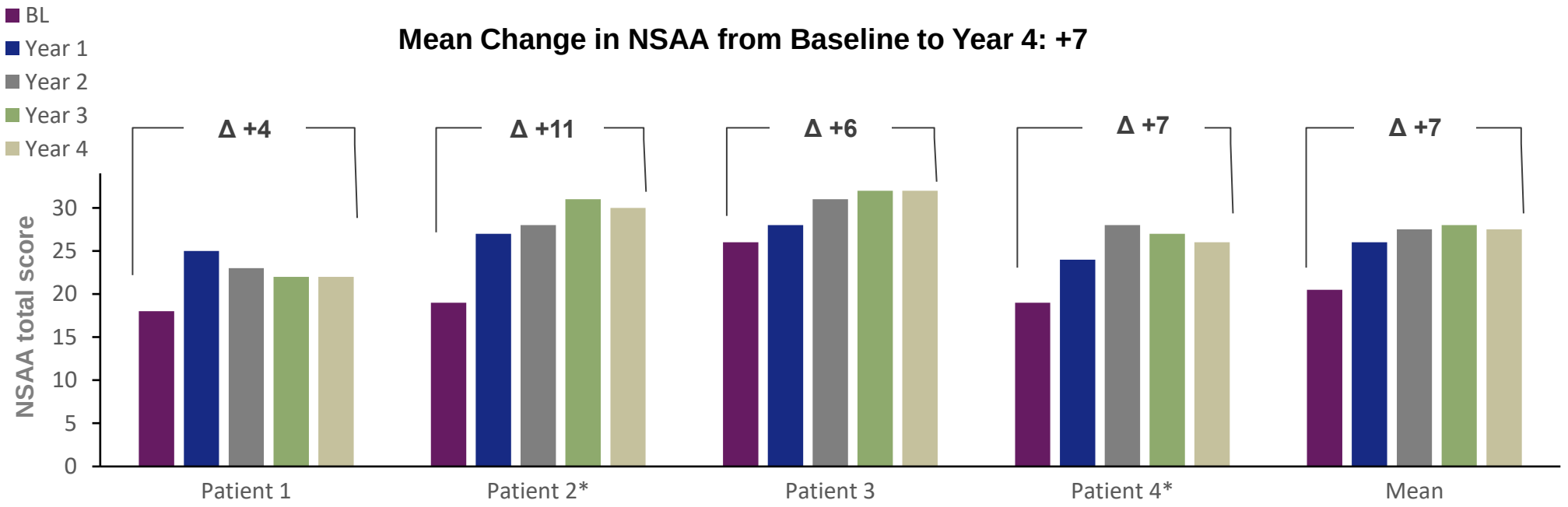
Source: Mendell, J. et al, ICNMD Conference 2022 and data on file.





1. 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.
*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.

Subjects in Study SRP-9001-101 demonstrated a mean increase of 7.0 points in total NSAA score from baseline to year 4



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.

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

1-year functional results compared to external control

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 intended commercial process 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)

Intended commercial process material

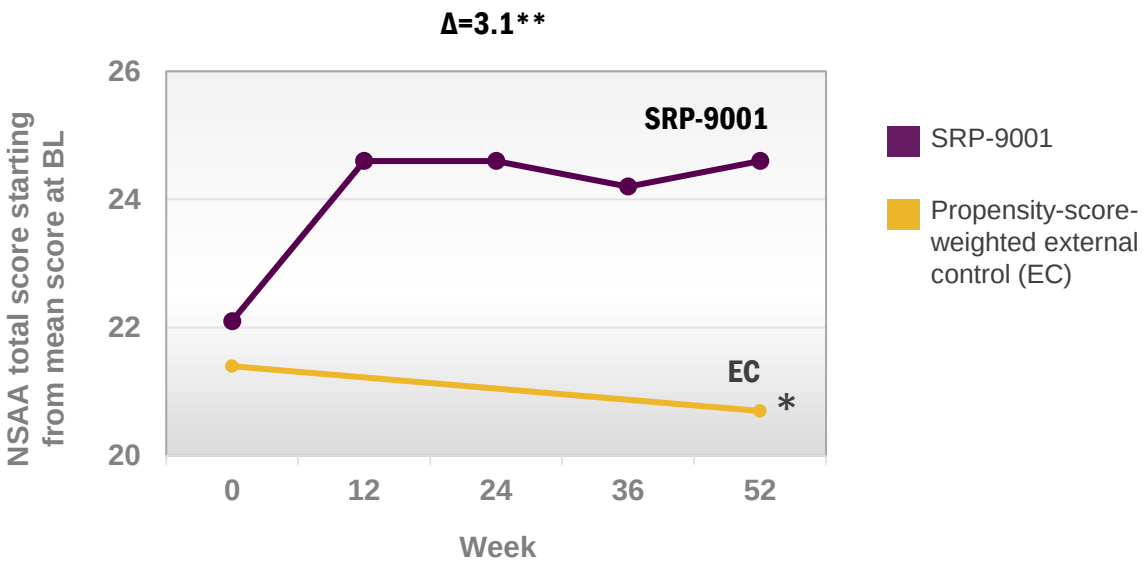
*The dose of delandistrogene moxeparvovec in Study 101 was 2.0×10^{14} vg/kg determined by supercoiled qPCR method (equivalent to 1.33×10^{14} vg/kg using qPCR with linear standard). †The intended target dose in Study 102 was 1.33×10^{14} vg/kg delandistrogene moxeparvovec IV infusion compared with placebo 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.

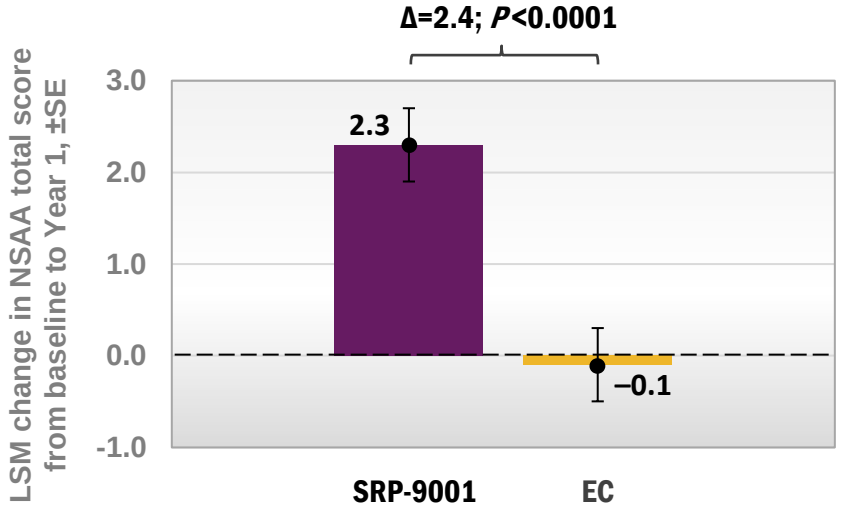
Integrated analyses: Statistically significant NSAA Total score compared to external control group at 52 weeks

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

Comparisons to EC data are not available for 100-meter Walk/Run and Time to Ascend 4 steps as noted on Slide 20.

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

Consistent NSAA improvement seen across multiple analysis sets

STUDY 102 Part 2 20 patients	Integrated Efficacy Analysis at Target Dose 52 patients	STUDY 103 20 patients		STUDY 102 Part 1 20 patients	STUDY 101 4 patients
2-point NSAA improvement compared to external control group at 1 year (mean).	**2.4-point** NSAA change from baseline compared to external control over 1 year (least square means).	**3.2-point** NSAA change from baseline compared to external control over 1 year (least square means).	**Statistically significant** difference demonstrated in time-to-rise and 10-meter walk/run compared to external control group at 1 year (means).	**5-point** NSAA difference from baseline compared to external control at 2 years (median).	**9.4-point** NSAA change from baseline compared to external control over 4 years (least square means).

Expression Data

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)	**74.3**	**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

ELEVIDYS safety summary

- The most common adverse reactions (incidence ≥ 5%) reported in clinical studies were vomiting, nausea, liver function test increased, pyrexia, and thrombocytopenia.
- Adverse reactions (incidence ≥5%) following treatment with ELEVIDYS in clinical studies:

Adverse Reactions	ELEVIDYS (n=85) %
Vomiting	61
Nausea	40
Liver function test increased[a]	37
Pyrexia	24
Thrombocytopenia[b]	12

a Includes: AST increased, ALT increased, GGT increased, GLDH increased, hepatic enzyme increased, transaminases increased, blood bilirubin increased
b Transient, mild, asymptomatic decrease in platelet counts

- Adverse reactions occurring in ELEVIDYS-treated subjects and at least 10% more frequently than in placebo in Study 1, Part 1:

Adverse Reactions	ELEVIDYS (N=20) %	Placebo (N=21) %
Vomiting	65	33
Nausea	35	10
Liver function test increased[a]	25	0
Pyrexia	20	5

a Includes: AST increased, ALT increased, GGT increased, GLDH increased, hepatic enzyme increased, transaminases increased, blood bilirubin increased

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.
*For the integrated safety data, the clinical cut-off dates were April 26, 2022 for Study 101, April 1, 2022 for Study 102, and April 6, 2022 for ENDEAVOR.
**This event has been disclosed previously.

ECHO, echocardiogram; IV, intravenous; MRI, magnetic resonance imaging; SAE, serious adverse event; SUSAR, suspected unexpected serious adverse reaction.

Clinical data show ELEVIDYS has the potential to change Duchenne disease progression

Data from SRP-9001-101, 102, 103 and integrated analysis (SRP-9001-101, 102, 103) demonstrated robust expression and consistent results

- **Clinical Impact**
  - **SRP-9001-103 (ENDEAVOR):** Data generated from intended commercial process 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 propensity matched 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
  - ELEVIDYS is contraindicated in patients with any deletion in exons 8 and/or 9

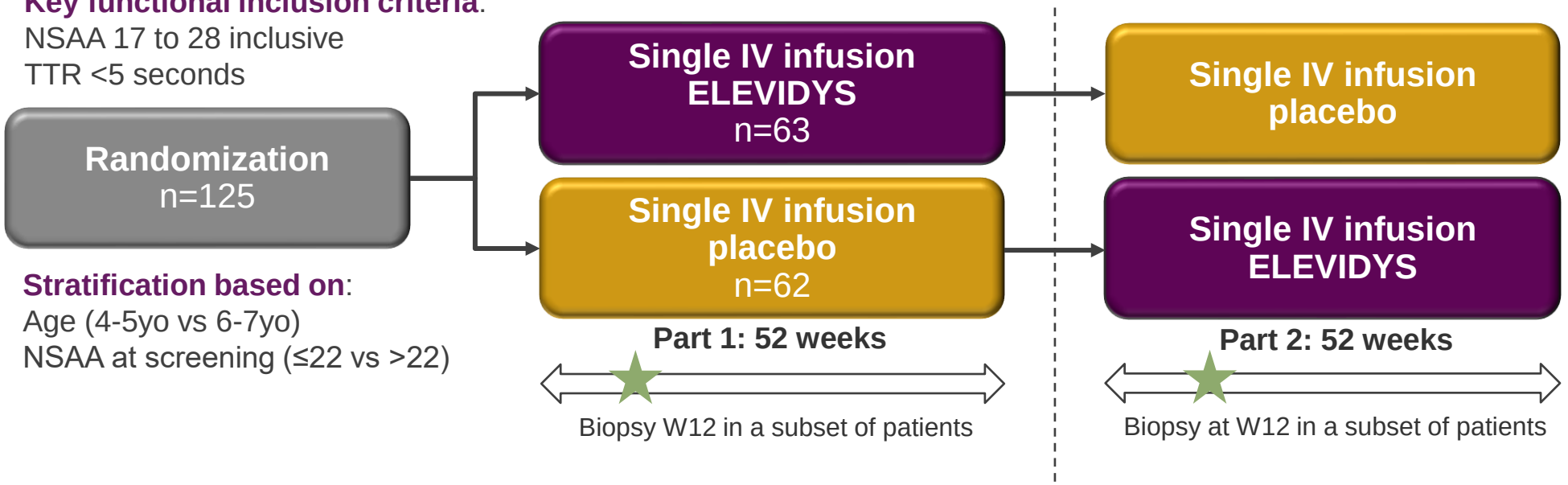
*See slides 36 and 37 for most common treatment-emergent adverse events (TEAEs) and safety profile.

EMBARK (Study SRP-9001-301) Top Line Results

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



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

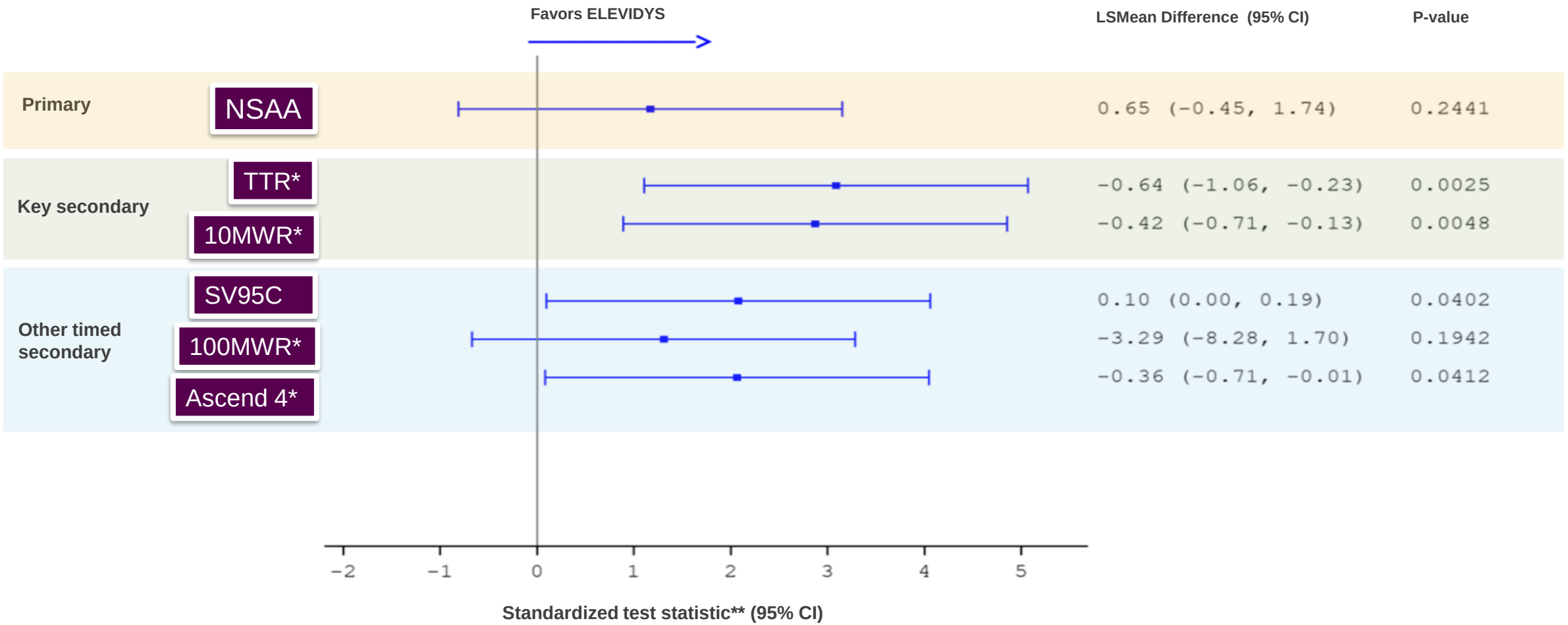
Primary endpoint:
- Change in NSAA total score from Baseline to Week 52

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

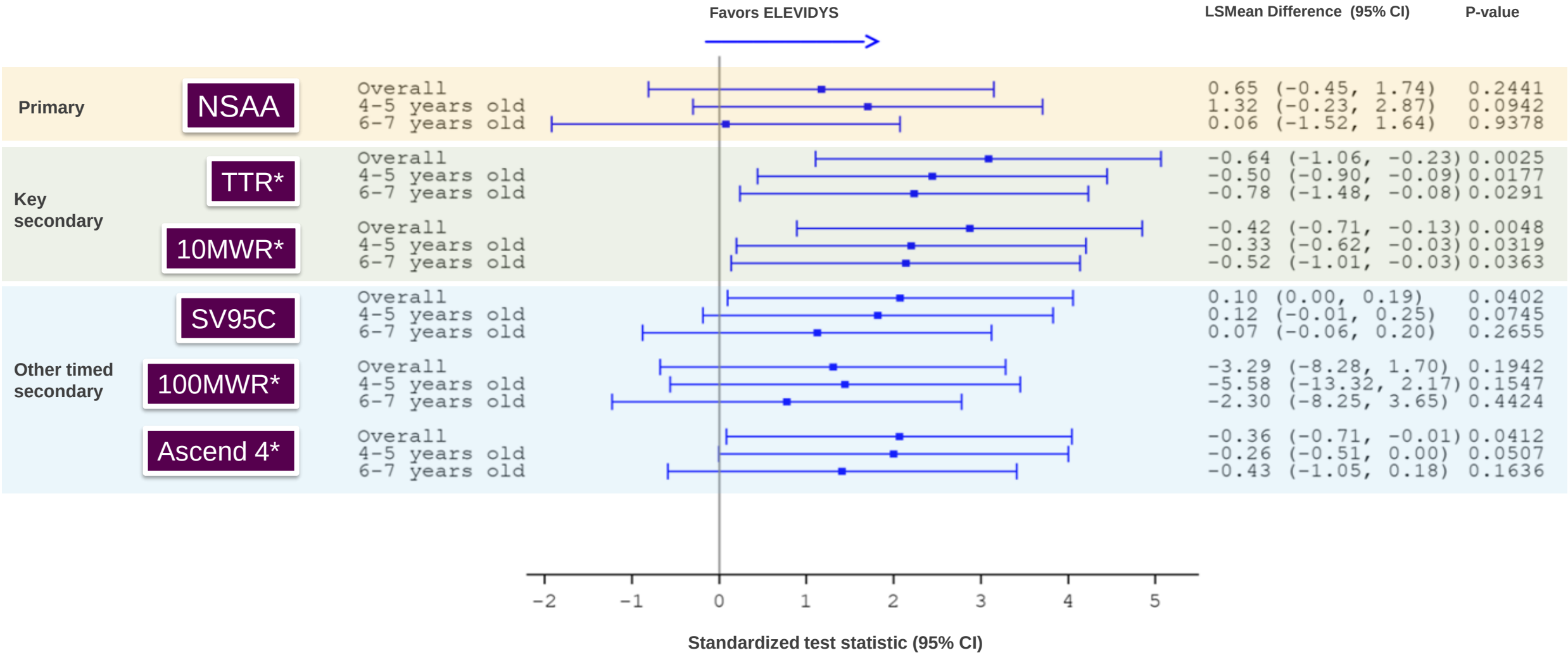
Results favor treatment with ELEVIDYS on all endpoints

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

Summary

- We believe the data from EMBARK exceeded 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
- No new safety signals were observed

ENVISION (Study SRP-9001-303) Trial Design

ENVISION (Study SRP-9001-303): Trial design

Design: Phase 3, multinational, randomized, double-blind, placebo-controlled study; 11 countries

Objectives: Evaluate the safety and efficacy of SRP-9001 in non-ambulatory and ambulatory individuals with Duchenne

Participants: 148 boys with Duchenne

Dose: Weight-based dosing, 1.33×10^{14} vg/kg up to 70kg

Selected inclusion criteria:

- Cohort 1 only: Non-ambulatory
- Cohort 2 only: Ambulatory, ≥8 to <18 years
- Stable daily dose of oral corticosteroids for at least 12 weeks prior to screening
- Negative for rAAVrh74 antibodies
- A pathogenic frameshift mutation or premature stop codon contained between exons 18 and 79 (inclusive)

Selected exclusion criteria:

- Exposure to gene therapy, investigational medication, or any treatment designed to increase dystrophin expression within protocol specified time limits
- Abnormality in protocol-specified diagnostic evaluations or laboratory tests
- Presence of any other clinically significant illness, medical condition, or requirement for chronic drug treatment

Primary endpoint (Part 1):

- Change from baseline at Week 72 in the total score of PUL (performance of upper limb)

Selected secondary endpoints:

- Change from baseline in Forced Vital Capacity (FVC) at Week 72
- Change from baseline in Percent Predicted Peak Expiratory Flow (PEF) at Week 72
- Expression at Week 12 as measured by Western Blot and Immunofluorescence
- Change from baseline in upper extremity function to Week 72*
- Change from baseline in Global Circumferential Strain as measured by cardiac MRI at Week 72
- Change from baseline in the North Star Ambulatory Assessment (NSAA) total score at Week 72 (Cohort 2 only)
- Safety

*Patient-Reported Outcomes Measurement Information System (PROMIS®) tool

SRP-5051 (vesleteplirsen): Lead RNA (PPMO) Pipeline Program

SRP-5051: Next-generation RNA-based PPMO* candidate to treat Duchenne patients amenable to exon 51 skipping**

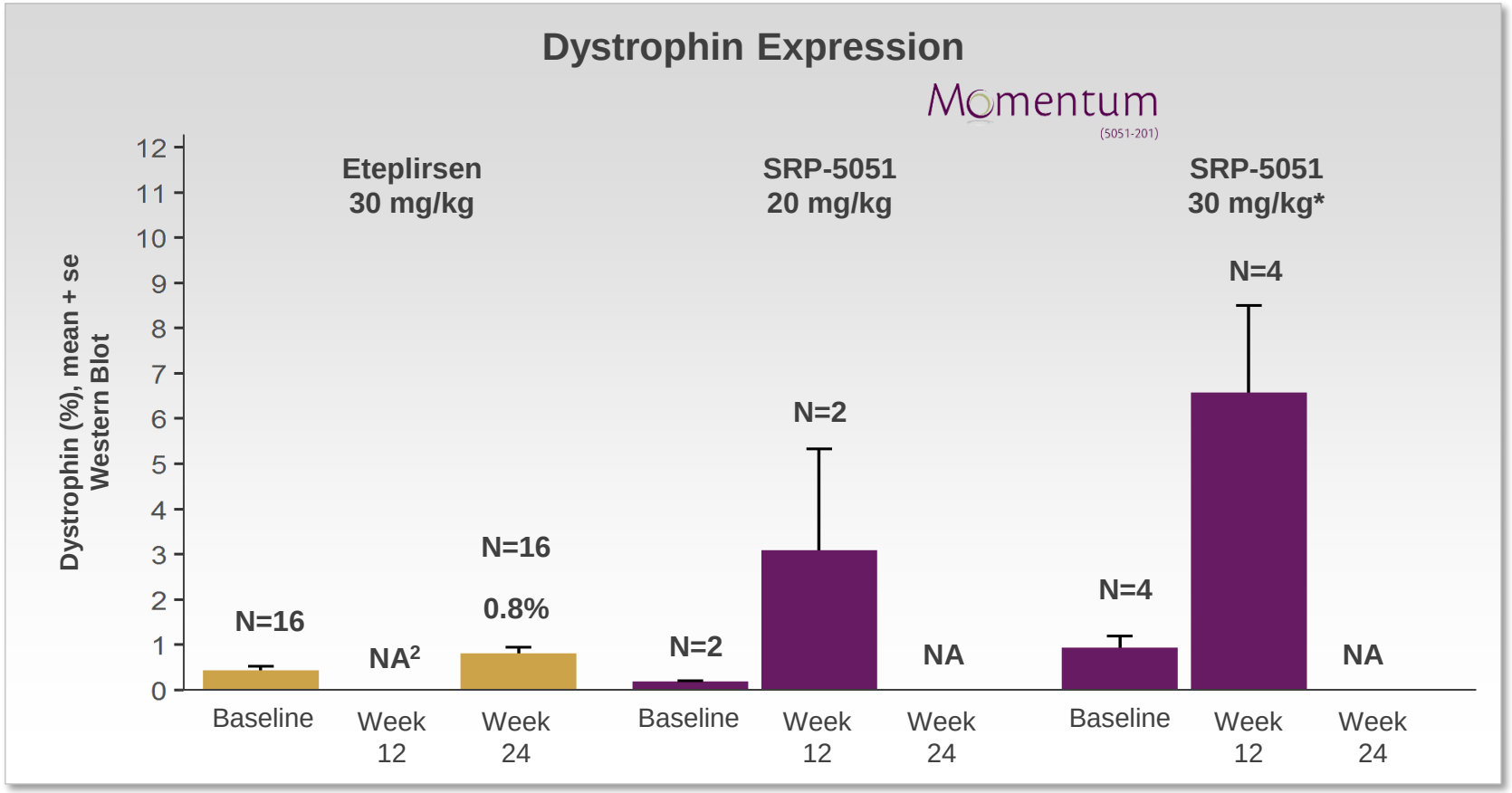
- Same precision genetic medicine backbone
- Conjugated peptide with the goal to increasing tissue penetration, exon skipping and dystrophin production
- Nonclinical data demonstrate delivery of PPMOs to all muscle, including the heart

*Peptide phosphorodiamidate morpholino oligomers (PPMO)
**Approximately 13% of patients with Duchenne are amenable to exon 51 skipping

MOMENTUM (Study SRP-5051-201, Part A) Clinical Results

Clinical results: SRP-5051 achieved higher dystrophin vs. eteplirsen

Next-generation technology enhances tissue penetration leading to greater exon skipping and dystrophin production



- Part A of 5051-201 complete
- 30 mg/kg at 12 weeks: 18x increase in exon skipping & 8x increase in dystrophin vs. eteplirsen at 24 weeks
  - Predicted dystrophin >10% expression over time with monthly dosing of SRP-5051
  - Patient receiving the most doses of SRP-5051 had the highest dystrophin expression
- Benefit/risk supports continued clinical development

~8x increase in dystrophin at 12 weeks vs. eteplirsen at 24 weeks[1]

1. Comparative data produced with the same analytical methods using biopsies obtained from Part A of Study 5051-201 MOMENTUM and Study 4658-202 PROMOVI.
2. NA Not Applicable, data not collected at these time points.

*Target biopsy was at 12 weeks. Patient 1 had 5 doses -19 weeks from baseline to biopsy.

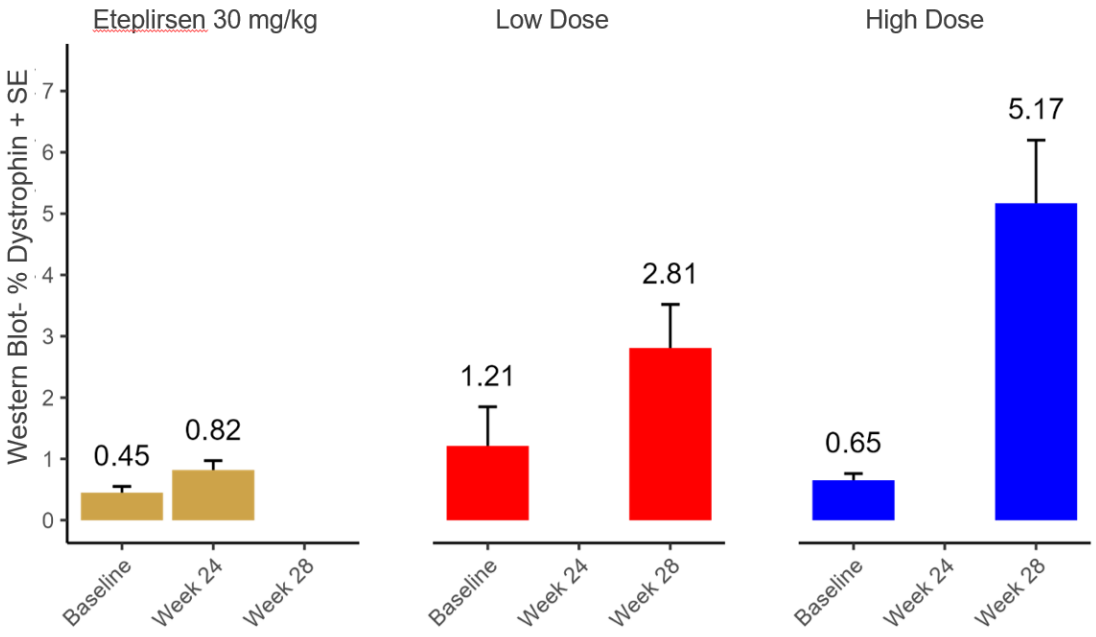
MOMENTUM (Study SRP-5051-201, Part B) Clinical Results

MOMENTUM (Study SRP-5051-201)

- Global trial evaluating SRP-5051 in patients with Duchenne amenable to exon 51 skipping
- Dystrophin protein levels in skeletal muscle tissue following treatment with SRP-5051 were assessed, as well as safety and tolerability
- Doses (administered every 4 weeks):
  - High dose: ~30 mg/kg
  - Low dose: ~20 mg/kg
- MOMENTUM (Study SRP-5051-201, Part B) enrolled 40 patients, 50% ambulant and 50% non-ambulant, ages 8 to 21 in the United States, Canada, and Europe
  - Primary outcome: Change from baseline in dystrophin protein level at week 28
- Patients dosed in MOMENTUM Part A, who met the entrance criteria, were eligible to participate in Part B
- Throughout MOMENTUM Part B we continued to administer prophylactic magnesium supplementation and/or adjust dose to manage hypomagnesemia

SRP-5051 showed mean dystrophin expression of 5.17% at the high dose at week 28

Data also demonstrated a 12.2x increase vs. eteplirsen

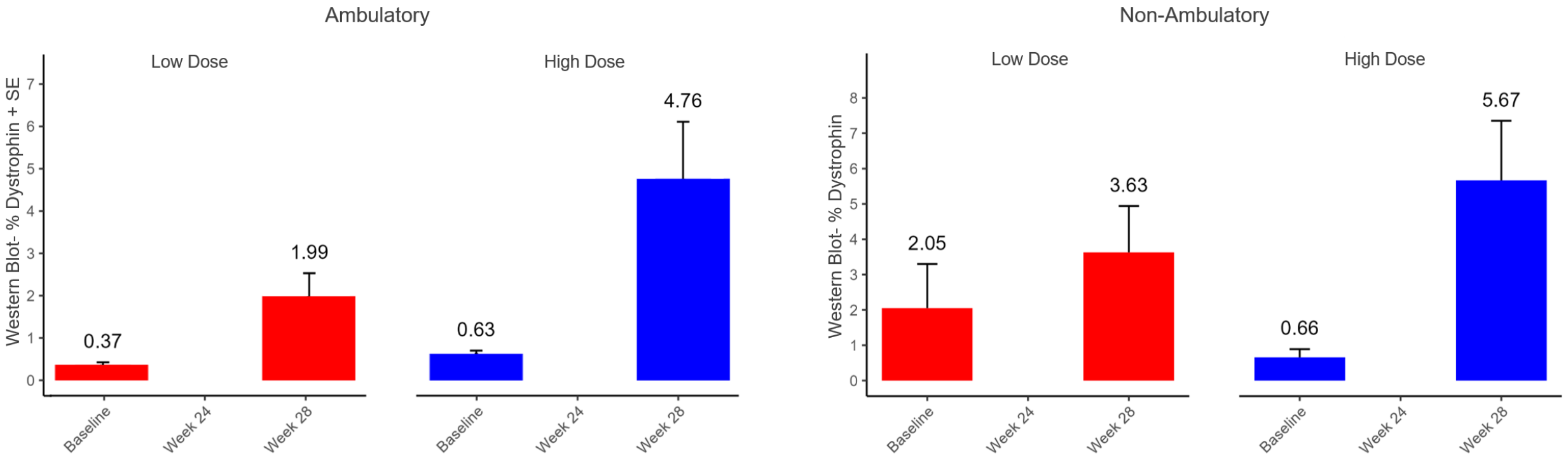


	Mean (SD) post baseline (%)	Mean (SD) change from baseline (%)	Multiplier vs. eteplirsen change from baseline
Eteplirsen (n=16)	0.82 (0.60)	0.37 (0.66)	--
SRP-5051 Low dose (n=20)	2.81 (3.19)	1.60 (2.21) P = 0.0012	**4.3x**
SRP-5051 High dose (n=20)	5.17 (4.62)	4.53 (4.78) P < 0.0001	**12.2x**

SE = Standard Error; SD = Standard Deviation

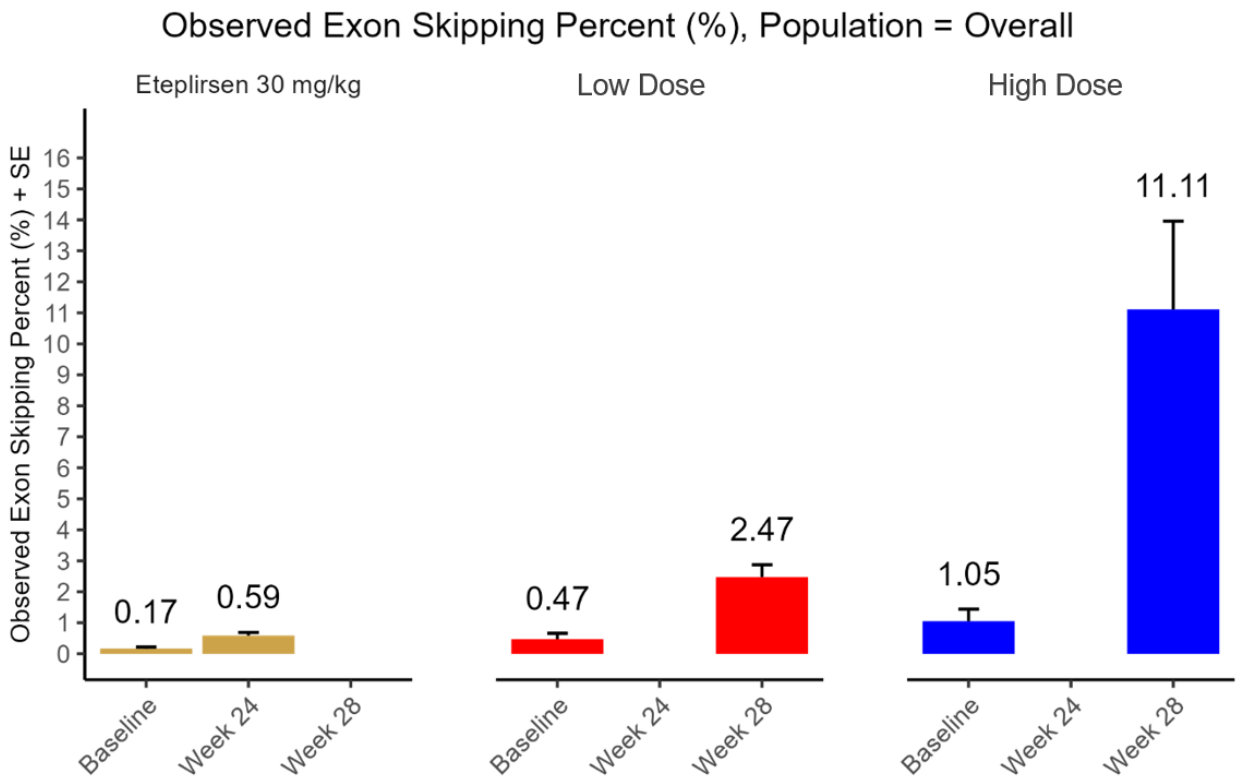
Both doses showed a change from baseline with statistically significant values

Similar expression levels observed in the ambulatory (n=21) and non-ambulatory (n=19) patient populations



SRP-5051 showed mean exon skipping of 11.11% at the high dose at week 28 (n=20)

Data also demonstrated 24.6x increase vs. eteplirsen

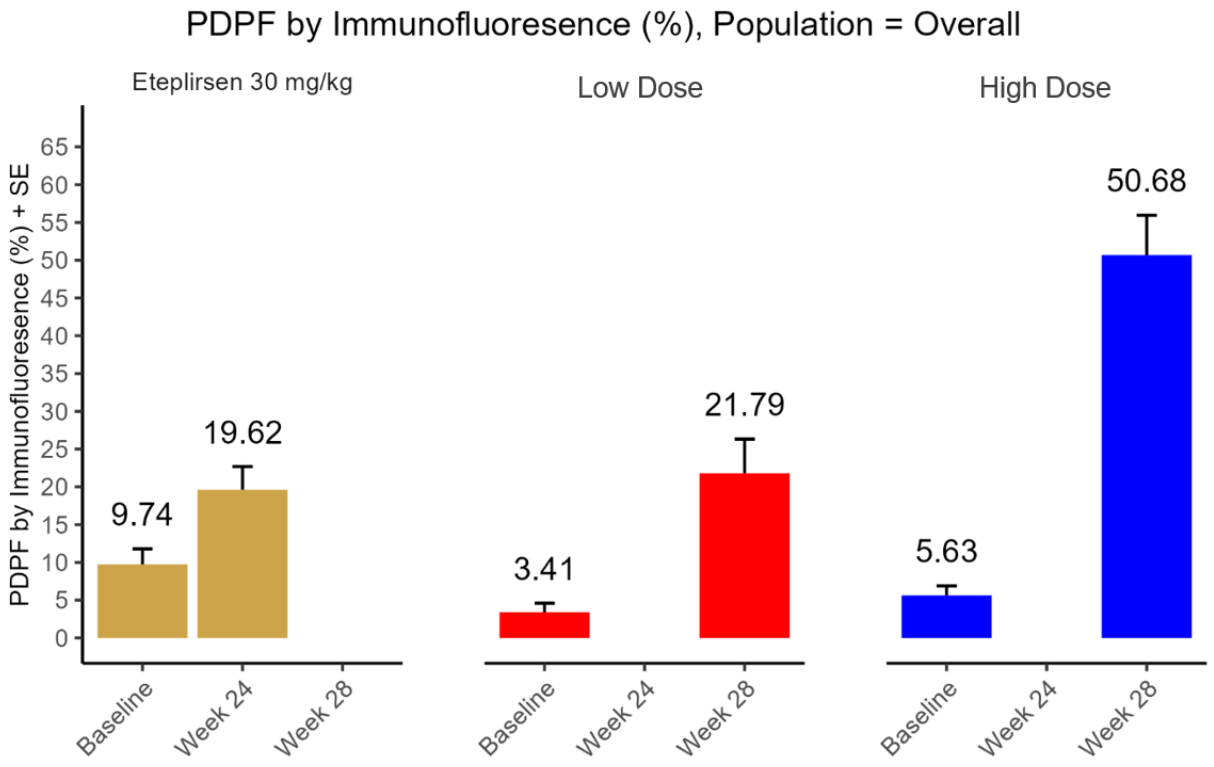


Exon skipping	Mean post baseline (%)	Mean change from baseline (%)	Multiplier vs. eteplirsen change from baseline
Eteplirsen (n=16)	0.59	0.41	--
SRP-5051 Low dose (n=19*)	2.47	2.00	**4.9x**
SRP-5051 High dose (n=20)	11.11	10.07	**24.6x**

*1 patient had degraded sample

SRP-5051 showed mean PDPF of 50.68% at the high dose at week 28

Data also demonstrated 4.6x increase vs. eteplirsen



	Mean post baseline (%)	Mean change from baseline (%)	Multiplier vs. eteplirsen change from baseline
Eteplirsen (n=16)	19.62	9.88	--
SRP-5051 Low dose (n=20)	21.79	18.38	**1.9x**
SRP-5051 High dose (n=20)	50.68	45.05	**4.6x**

Safety Results

MOMENTUM (SRP-5051-201, Part B): Safety experience overview

Adverse Event Summary (mean 12 months of dosing)

	Low Dose (N=32) n (%)	High Dose (N=29) n (%)	Overall (N=62) n (%)
Subjects with Treatment-Related TEAEs	31 (96.9%)	28 (96.6%)	60 (96.8%)
Treatment-Related AEs in > 5% of subjects			
Hypomagnesemia	30 (93.8%)	28 (96.6%)	59 (95.2%)
Hypokalemia	12 (37.5%)	14 (48.3%)	26 (41.9%)
Nausea	2 (6.3%)	3 (10.3%)	5 (8.1%)
Vomiting	2 (6.3%)	2 (6.9%)	4 (6.5%)
Glomerular filtration rate decreased	1 (3.1%)	3 (10.3%)	4 (6.5%)
Treatment-Related SAEs			
Hypomagnesemia	1 (3.1%)	3 (10.3%)	4 (6.5%)
Hypokalemia	1 (3.1%)	2 (6.9%)	3 (4.8%)
Treatment-Related Discontinuations	0	0	0

Source: Table 14.3.1.1.1 Overall Summary of Treatment-Emergent Adverse Events Safety Set – Part B
Data Cutoff Date: 02-NOV-2023

Summary and next steps

Efficacy

- Achieved study's primary endpoint
- Demonstrated a statistically significant increase in dystrophin expression at both doses
- At the high dose at week 28, SRP-5051 showed dystrophin production 12.2x higher than eteplirsen
- SRP-5051 demonstrated mean dystrophin expression of 5.17% at the high dose at 28 weeks

Safety

- The data support a positive benefit-risk profile for SRP-5051
- Throughout MOMENTUM Part B, we continued to administer prophylactic magnesium supplementation and/or adjust dose to manage hypomagnesemia
- No treatment-related discontinuations occurred in the study

Next Steps

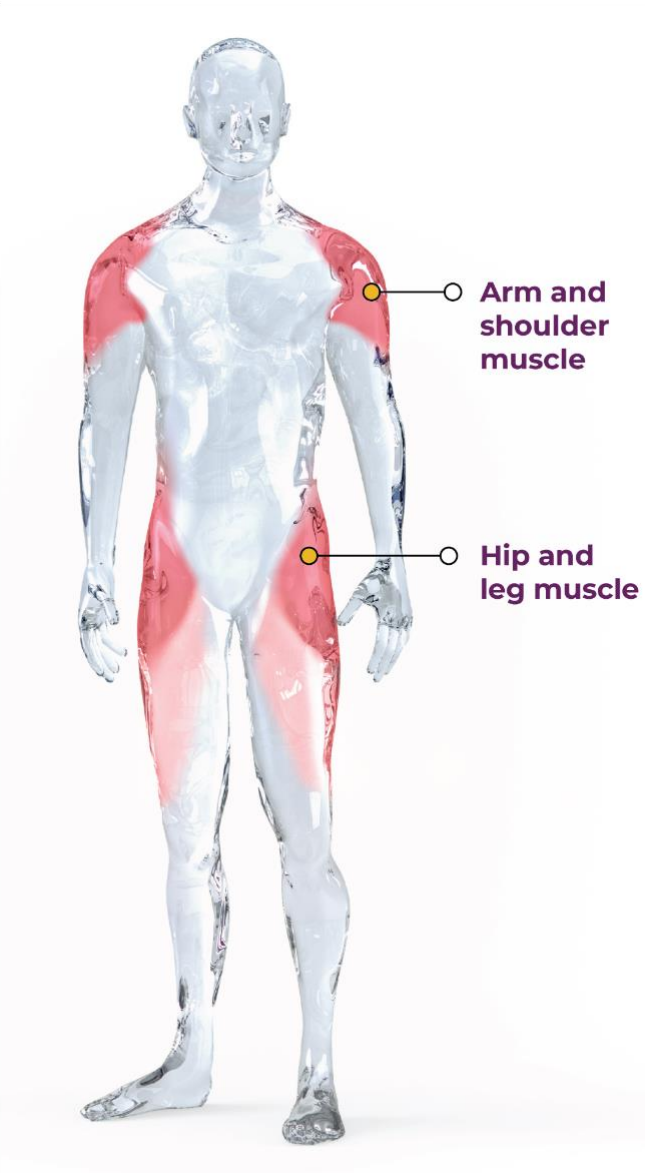
- Planning a meeting with FDA to discuss accelerated approval, expected timing Q3 2024
- Study remains ongoing

Limb-girdle muscular dystrophy (LGMD) Portfolio

Leading the way in LGMD

Approximate global prevalence of LGMDs as a group is 1.63 per 100,000[1]*

- The LGMDs are a group of inherited neuromuscular diseases that all cause progressive muscle weakness, the onset of disease can occur from childhood to adulthood[4]
- As a group LGMDs are the 4th most common muscular dystrophy
- Over 30 subtypes exist[2] with both genders affected equally[3]

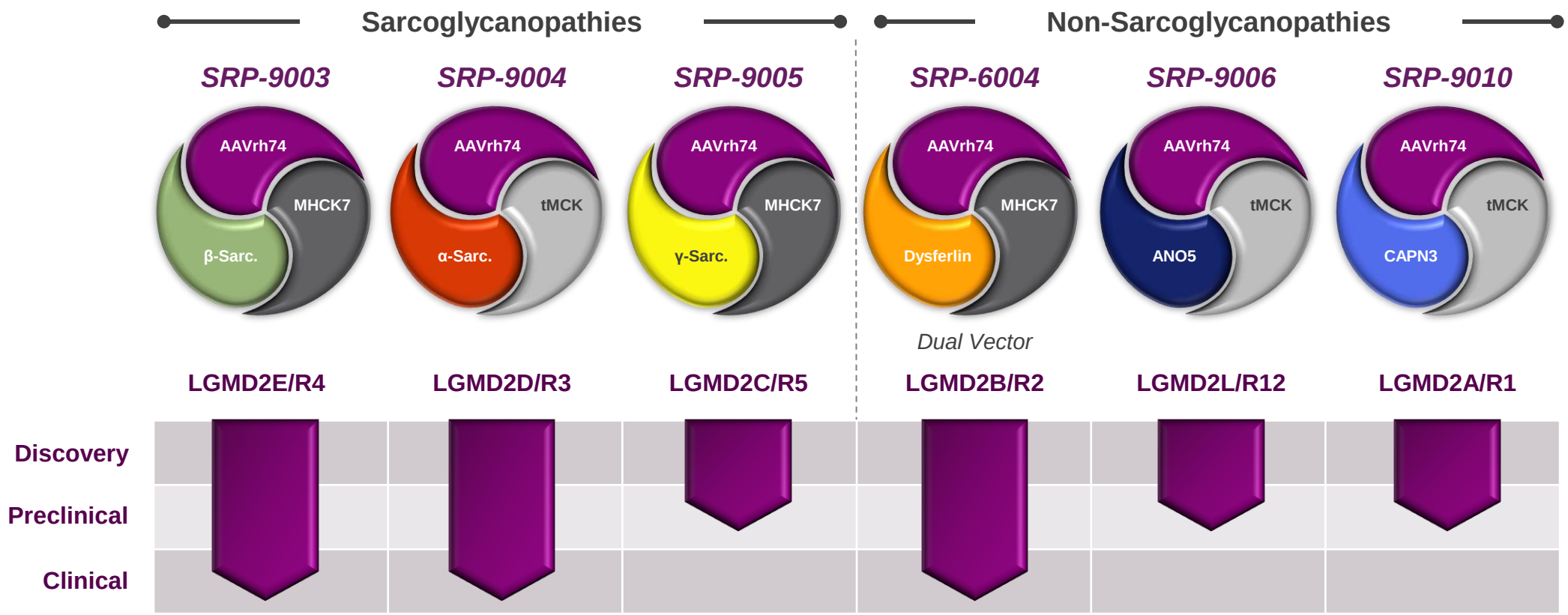


*Prevalence estimates range from 0.56 to 5.75 per 100,000.

1. Liewluck T, Milone M, et al. Untangling the complexity of limb-girdle muscular dystrophies. Muscle Nerve. 2018;58(2):167-177.
2. Murphy AP, Straub V. The Classification, Natural History and Treatment of the Limb Girdle Muscular Dystrophies. *J Neuromuscular Diseases*. 2015;2(s2):S7-S19.
3. Muscular Dystrophy Association. Limb-girdle muscular dystrophy (LGMD). Accessed Jan 2020.
4. Liewluck T, Milone M, et al. Untangling the complexity of limb-girdle muscular dystrophies. Muscle Nerve. 2018;58(2):167-177.

Market leading gene therapy portfolio in LGMD

CMC progress continues for sarcoglycanopathies; expect multiple clinical trial starts over next two years



Steady stream of gene therapy candidates covering >70% of known LGMD patients[1]

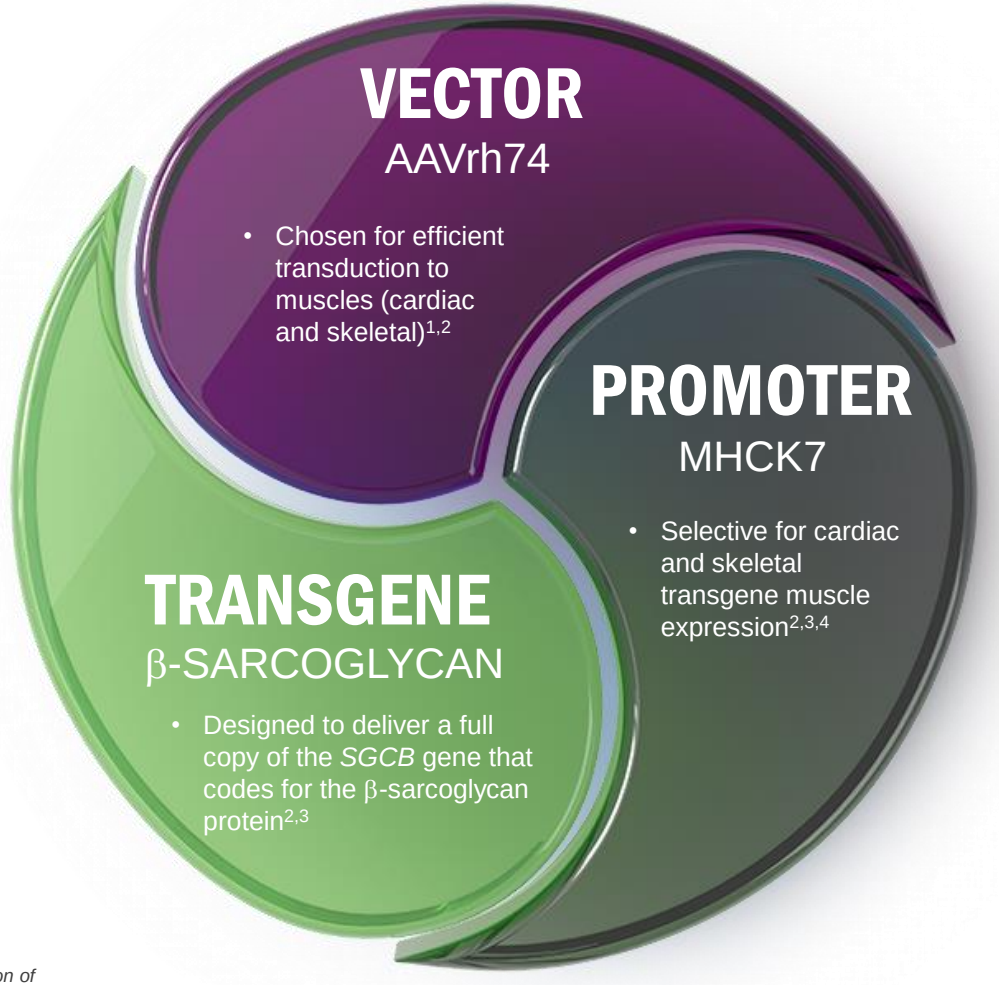
1. Taghizadeh E, Rezaee M et al. *J Cell Physiol*. 2019;234(6):7874-7884.

SRP-9003 (bidridistrogene xeboparvovec): Gene therapy for Limb Girdle Muscular Dystrophy type 2E/R4 (beta-sarcoglycanopathy)

SRP-9003 is an investigational therapy and has not been reviewed or approved by any regulatory authority.

Sarepta's gene therapy engine at work

Lead LGMD clinical development program, SRP-9003 in LGMD2E/R4



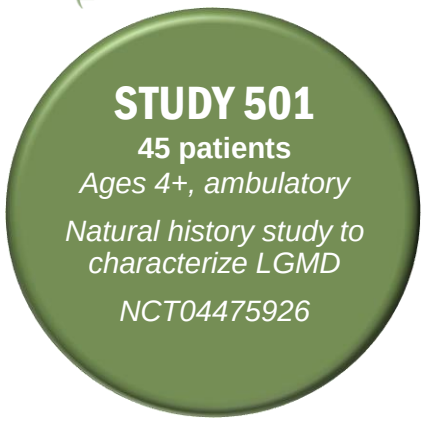
1. Chicoine LG, et al. Vascular Delivery of rAAVrh74.MCK.GALGT2 to the Gastrocnemius Muscle of the Rhesus *Macaque Stimulates the Expression of Dystrophin and Laminin α2 Surrogates.* Mol Ther. 2014;22(4):713-724.
2. Pozsgai ER, et al. Systemic AAV-Mediated b-Sarcoglycan Delivery Targeting Cardiac and Skeletal Muscle Ameliorates Histological and Functional Deficits in LGMD2E Mice. *Mol. Ther*. 2017 Apr 5;25(4):855-869.
3. Mendell JR, et al. Assessment of Systemic Delivery of rAAVrh74.MHCK7.micro-dystrophin in Children With Duchenne Muscular Dystrophy: A Nonrandomized Controlled Trial. *JAMA Neurol*. 2020 Jun 15;77(9):1-10.
4. Salva MZ, et al. Design of tissue-specific regulatory cassettes for high-level rAAV-mediated expression in skeletal and cardiac muscle. *Mol Ther*. 2007;15(2):320-329.

SRP-9003: Clinical development program

Gene construct (AAVrh74.MHCK7.SGCB) that aims to transduce skeletal and cardiac muscle, with the goal of delivering a gene that codes for the full-length β-sarcoglycan protein[1]

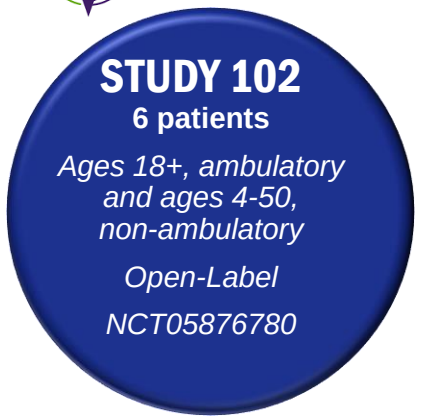
- Phase 1/2 study
- Cohort 1 (1.85 x 10^{13} vg/kg)[a]
- Cohort 2 Cohort 2 (7.41 x 10^{13} vg/kg)[b]
- Primary outcome: Safety
- Secondary outcome: Change in β-sarcoglycan protein expression from baseline to week 8*
- One-year clinical results published in *Nature Medicine*

JOURNEY



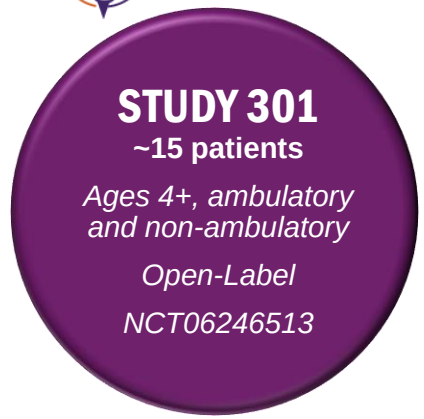
- Natural history
- Evaluating pulmonary and skeletal muscle function
- Fully enrolled
- All LGMD2E/R4 patients
- Total LGMD2E/R4 patients enrolled: 45

VOYAGENE



- Phase 1 study
- Cohort 1 (ambulatory)
- Cohort 2 (non-ambulatory)
- Primary outcomes: Safety and change in β-sarcoglycan expression from baseline to day 60

EMERGENE



- Phase 3 study
- 6-month natural history lead-in
- Primary outcome: Expression of β-sarcoglycan 60 days after dosing
- Other outcomes: Functional measures through month 60; and safety

CLINICAL SUPPLY INTENDED COMMERCIAL PROCESS MATERIAL NATURAL HISTORY

*Based on pre-clinical studies, the goal was to achieve expression levels of ≥20%.

1. Rodino-Klapac L, et al. Systemic Gene Transfer with rAAVrh74.MHCK7.SGCB Increased β-sarcoglycan Expression in Patients with Limb Girdle Muscular Dystrophy Type 2E (LGMD2E). WMS 2020.

[a]1.85×10^{13} vg/kg measured using linear reference plasmid DNA qPCR; supercoiled reference plasmid DNA equivalent is 5×10^{13} vg/kg

[b]7.41×10^{13} vg/kg measured using linear reference plasmid DNA qPCR; supercoiled reference plasmid DNA equivalent is 2×10^{14} vg/kg

Study SRP-9003-101: Clinical data summary

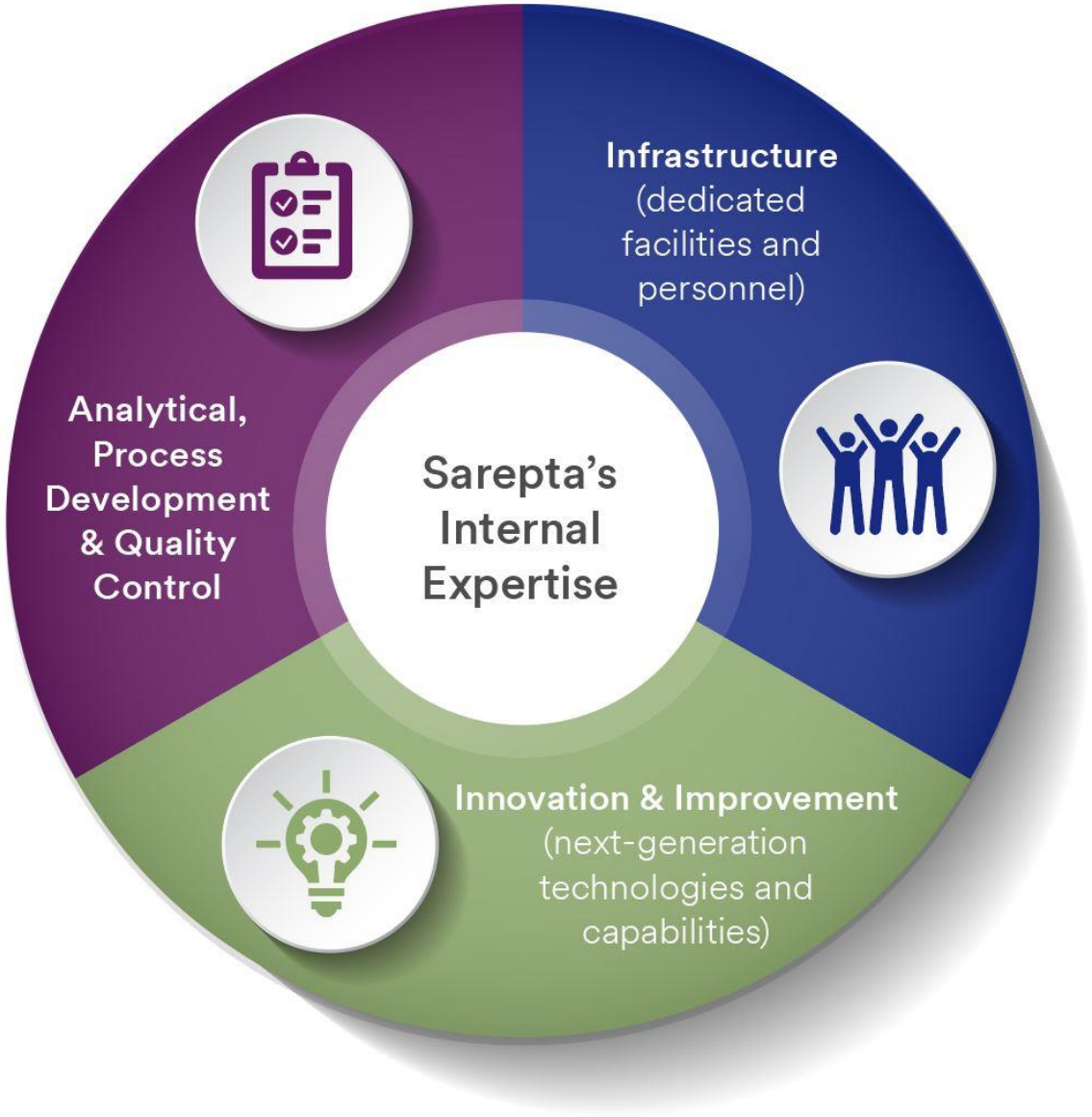
	1	2	3	4	5
QUESTION[1]	What was the safety and tolerability experience with SRP-9003?	Is the transgene DNA inside muscle cells?	Is the desired protein made?	Is the protein at the cell membrane?	Is muscle function improved?
EXPERIMENT[2,3]	SAFETY	VECTOR GENOME COPIES/NUCLEUS (ddPCR)	WESTERN BLOT	IMMUNO-FLUORESCENCE	FUNCTIONAL OUTCOMES
	• Systemic administration of SRP-9003 is well tolerated to date with up to 3 years of follow-up for Cohort 1 and 2 years for Cohort 2 • No unexpected immunologic responses in these patients	**Copies per nucleus** • **Cohort 1:** D60 NE[a]; Y2 0.46 • **Cohort 2:** D60 2.26; Y2 0.52	**SGCB expression** • **Cohort 1:** D60 36%; Y2 54% • **Cohort 2:** D60 62%; Y2 60%	**Percentage of cells with protein** **Percentage of SGCB-positive fibers:** • **Cohort 1:** D60 51%; Y2 47% • **Cohort 2:** D60 72%; Y2 63% **Intensity of fluorescent signal:** • **Cohort 1:** D60 47%; Y2 35% • **Cohort 2:** D60 73%; Y2 44% Rescue of membrane localization of SGCA, SGCG, and SGCD proteins and reconstitution of the sarcoglycan complex within the DAPC	**NSAD** Mean (SD) NSAD score vs baseline (BL): • **Cohort 1:** 48 (5.7) Y3 vs 43 (4.4) BL • **Cohort 2:** 41 (0) Y2 vs 39 (2.1) BL LS mean change from baseline of treated patients compared with natural history cohort at Y3: • **5.9-point difference** (95% CI, -1.5, 13.3)

[a]Mean (SD) qPCR value of day 60 cohort 1 was 0.59 (0.4).
BL=baseline; D=day; DAPC=dystrophin-associated protein complex; ddPCR=droplet digital PCR; LS=least squares;
NE = not estimated; NSAD=North Star Assessment of Limb-girdle type Muscular Dystrophies; SGCA=a-sarcoglycan; SGCB=β-sarcoglycan; SGCD=δ-sarcoglycan; SGCG=γ-sarcoglycan; Y=year.

1. Asher DR, et al. *Expert Opin Biol Ther*. 2020;20:263-74.
2. Rodino-Klapac, et al. Safety, β-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of Bidridistrogene Xeboparvovec in Limb-Girdle Muscular Dystrophy Type 2E/R4. Poster presented at 27th International Hybrid Annual Congress of the World Muscle Society; October 11–15, 2022; Halifax, Nova Scotia, Canada.
3. Rodino-Klapac,et al. Safety, β-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of rAAvrh74.MHCK7.hSGCB in LGMD2E/R4. Presentation presented at the International Congress on Neuromuscular Diseases, July 5–9, 2022; Brussels, Belgium.

Setting the Standard in Gene Therapy Manufacturing

Sarepta's manufacturing expertise



Internal gene therapy capabilities complimented by partnerships: Meeting demand to support launch of ELEVIDYS

Dedicated Sarepta Facilities and Capabilities

Analytical, Process Development & Quality Control

- Vector & drug product development
- Non-clinical tox manufacturing
- Fully equipped AD/QC labs
- Validated methods for tittering/release

Investments in FTEs and Infrastructure

- >30k ft^2 facilities in Andover and Burlington, MA
- >300 dedicated staff for technical operations and manufacturing
- Expanding GMP manufacturing footprint in Bedford, MA facility

Continued Innovation and Improvement

- Approximately 140,000 sq. ft. for early research and development, as well as process development (Columbus, OH)
- Developing next-gen technologies to improve efficiencies and reduce COGS (e.g., suspension manufacturing process)

External Partnership Overview

External Partner	Description	Status
aldevron®	Plasmid Production	Dedicated capacity for Sarepta portfolio
Catalent	Vector Production (Drug Substance & Drug Product)	Dedicated space for Sarepta
PPD®	Analytical Testing	Dedicated FTEs to support Sarepta programs

Hybrid approach will drive competitive costs with continual improvements to drive upside

Gene Editing: Early-stage Programs

LOUISE RODINO-KLAPAC, Ph.D.
EVP, Head of R&D, Chief Scientific Officer
Sarepta Therapeutics, Inc.

GARY CHARBONNEAU
SVP, General Manager, Head R&ED
Sarepta Therapeutics, Inc.

CHARLES GERSBACH, M.D.
Consultant, Director, GEIC

GENETIC THERAPIES CENTER OF EXCELLENCE (GTCOE)
COLUMBUS, OH

Discovery, Supportive Non-clinical Research
Identify target diseases/genes, and assess viability for clinical development

Translational & Clinical Development
Develop and run critical clinical assays for immunogenicity, efficacy, safety

Vector, Process & Analytical Development
Optimize and produce vector, and drive early-stage analytical development activities

GENE EDITING INNOVATION CENTER (GEIC)
DURHAM, NC

Differentiated Scientific Approach
- Proprietary dual cut strategy for predictable and accurate editing
- Potential to mitigate safety and durability challenges facing competing approaches

Innovating to Optimize Delivery and Safety
- Investigating viral and non-viral delivery
- Designing novel cargo to limit adverse events

Addressing Diseases with High Unmet Need
- Developing approaches that may treat the majority of DMD patients
- Pursuing diseases that are difficult to treat with traditional gene replacement

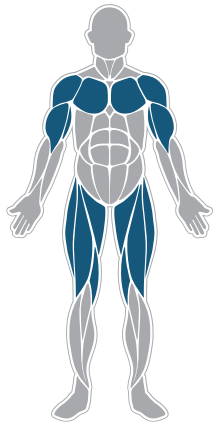
A Distinct Partnership Model and Future Innovations

Sarepta's strategy designed to enable sustainable long-term growth

Maximizing ELEVIDYS and in-line gene therapies

*Ensure every eligible Duchenne **patient can receive ELEVIDYS**, and maximize opportunity for in-line gene therapies*

Enable NMD expansion



*Enable **best-in-class genetic therapies** in neuromuscular space*

Progress to adjacencies

*Internal efforts and collaborations with **leading partners to tackle largest challenges in gene therapy**, and expand Sarepta's focus into attractive adjacencies*

MyoAAV program: Next-generation technology with applicability across multiple genetic-based diseases

- **MyoAAV Platform**
  - New approach holds promise to generate greater efficacy (i.e., restoration of muscle function) at lower therapeutic doses

- **Status**
  - License agreement with the Broad Institute of MIT and Harvard (MyoAAV for Duchenne, and other neuromuscular/cardiac indications)
  - Early Duchenne data in animal model studies published in *Cell* (2021) by the Broad Institute and sponsored by Sarepta Therapeutics

- **Next Steps**
  - Pre-clinical studies underway

Sarepta’s Mission

Armed with the most advanced science in genetic medicine, we are in a daily race to rescue lives otherwise stolen by rare disease.

At Sarepta, every day is another 24 hours to stand up for patients, advance technology, challenge convention and ***drag tomorrow into today***.

CHARLES
Living with Duchenne muscular dystrophy

Dragging tomorrow into today

#DraggingTomorrowIntoToday