2019 PPMD ANNUAL CONFERENCE

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Executive Vice President, R&D and Chief Medical Officer

June 29, 2019

CALEB
Living with Duchenne Muscular Dystrophy

A NEW ERA OF MEDICINE IS UPON US

SAREPTA THERAPEUTICS

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," "would," "should," "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 goal of treating 100% of individuals with DMD; exon skipping's goal to restore the reading frame, by skipping over an exon near the deletion; the expectation to have three RNA therapies on the market by 2020, serving ~30% of the DMD community; golodirsen's expected regulatory action date of August 2019, our plan to submit an NDA to the FDA for casimersen in mid-2019; our studies design and the plan to expand the 5051-201 study to other countries; our plans re PPMO next mutations and the expectation to have more clarity regarding PPMO in mid-2020; our pipeline and the potential benefits of our technologies and scientific approaches, including the potential benefits of PMO and the potential of PPMO to lead to more efficient dosing for patients.

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 could materially and adversely affect our business, results of operations and trading price. Potential known risk factors include, among others, the following: our data for our different programs, including golodirsen casimersen, micro-dystrophin and LGMD may not be sufficient for obtaining regulatory approval; 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; even if our programs result in new commercialized products, we may not achieve any significant revenues from the sale of such products; 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; 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, and 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 2018 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q 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.

SAREPTA THERAPEUTICS

OUR GOAL

Sarepta Therapeutics' goal is to develop life-changing precision genetic medicine to treat

100% of individuals with Duchenne muscular dystrophy

OUR CLINICAL EXPERIENCE IN DUCHENNE

As of Q1 2019
*Includes individual enrollments across all trials

SAREPTA'S PIPELINE FOR DUCHENNE MUSCULAR DYSTROPHY

RNA

GENE THERAPY

GENE EDITING

*Candidate received accelerated approval in the U.S., confirmatory studies required
**Other exon targets in development: 43, 44, 50, and 55

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SAREPTA'S PIPELINE FOR DUCHENNE MUSCULAR DYSTROPHY

SAREPTA'S PIPELINE FOR DUCHENNE MUSCULAR DYSTROPHY

SAREPTA THERAPEUTICS

PMO TECHNOLOGY FOR DUCHENNE MUSCULAR DYSTROPHY

GENE THERAPY

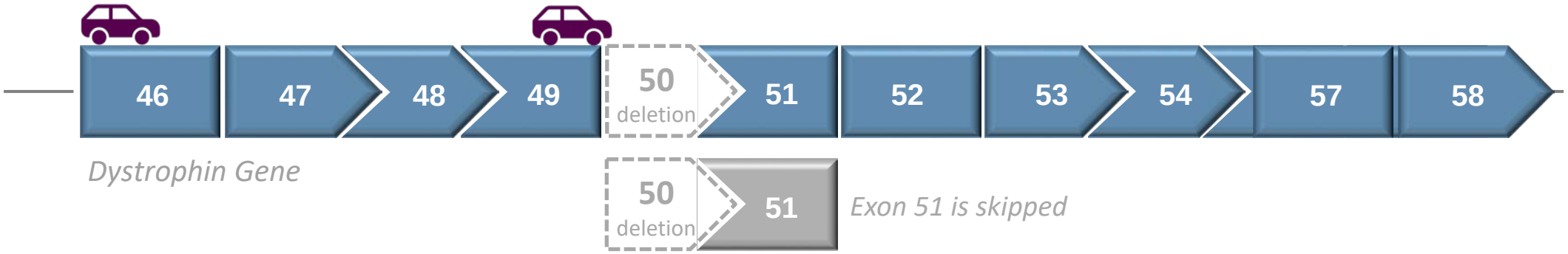
PMO

PPMO

PROBLEM: *Duchenne is caused when the dystrophin gene is incapable of making enough or any dystrophin protein – dystrophin is critical for muscle function*

PRECISION GENETIC TECHNOLOGY: *Phosphorodiamidate Morpholino Oligomers (PMOs) assemble in precise sequence, bind to the target, and direct the body to make a shortened functional form of the dystrophin protein*

GOAL OF EXON SKIPPING:



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THREE PMO PROGRAMS TO ADDRESS UP TO 30% OF INDIVIDUALS WITH DUCHENNE

ETEPLIRSEN:
PMO for skipping of Exon 51

Granted US Accelerated Approval in **2016**

GOLODIRSEN:
PMO for skipping of Exon 53

New drug application (NDA) submitted to FDA in December, 2018. Regulatory action date scheduled for **August 2019**.

CASIMERSEN:
PMO for skipping of Exon 45

Goal of submitting NDA to FDA in **mid-2019** based upon findings from interim muscle biopsies from patients in ESSENCE.

If successful, we will have 3 RNA-therapies by 2020, serving ~30% of the Duchenne community.

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Golodirsen and casimersen are investigational and not approved in the United States.

GOLODIRSEN CLINICAL DEVELOPMENT PROGRAM

PHASE I/II

Study 101[1] Part I
N=12 (golodirsen n=8; placebo n=4)
Age: 6-15 years
IV infusions, dose escalation

Study 101[1] Part II
N=24 (golodirsen)
Age: 6-15 years
IV infusions 30 mg/kg/week

PHASE III

Study 301 (ESSENCE)[2]
N=222 (golodirsen n=111; casimersen n=111)
Age: 7-13 years
IV infusions 30 mg/kg/week

Study 302[3]
N≈260 (open label golodirsen or casimersen)
Age: 7-23 years
IV infusions 30 mg/kg/week

Complete
Ongoing

Thank you to all of the individuals and families who have participated in Sarepta clinical trials.

Details of all trials can be found on ClinicalTrials.gov. Identifiers: 1. NCT02310906. 2. NCT02500381. 3. NCT03532542

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Golodirsen and casimersen are investigational and not approved in the United States.

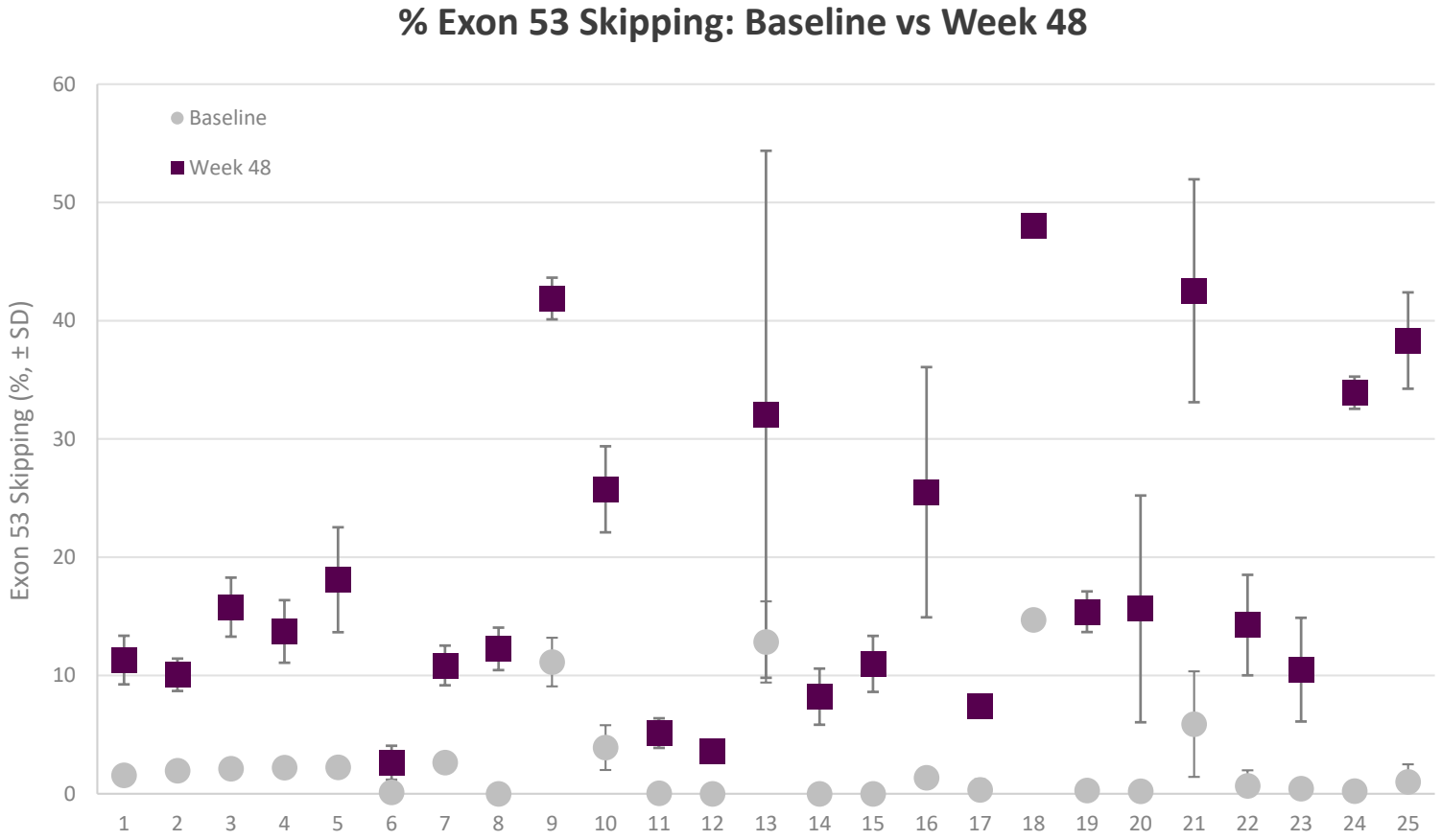
GOLODIRSEN CLINICAL DATA: WEEK 48 EXON SKIPPING RESULTS

Did exon skipping occur?

GENE THERAPY

PMO

PPMO



- Increased Exon 53 skipping observed in all patients
- Mean skipping increased from 2.59% at baseline to 18.95% at week 48

1. ClinicalTrials.gov Identifier: NCT02310906. 2. Frank D, Mercuri E, Servais L, et al. Presented at the 2019 American College of Medical Genetics and Genomics (ACMG) Annual Meeting, 4 April 2019, Seattle, WA.

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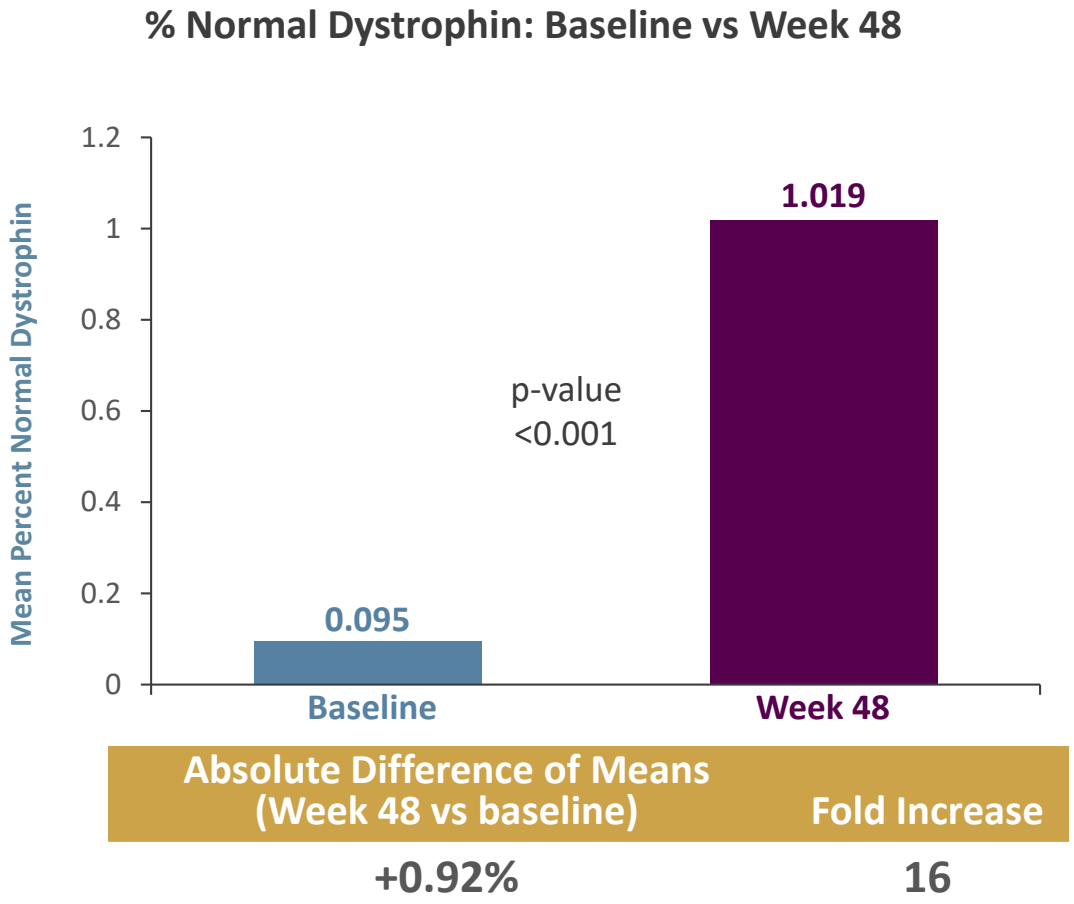
GOLODIRSEN CLINICAL DATA: WEEK 48 DYSTROPHIN RESULTS

Is dystrophin protein made?



- **Western Blot: ~16x mean per-patient fold increase in dystrophin (Baseline to Week 48)**
- IHC: Confirmed dystrophin localized to sarcolemma (not shown)

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GOLODIRSEN CLINICAL DATA: SAFETY (4053-101 PARTS 1 AND 2)

- All patients reported at least 1 adverse event after beginning treatment. The majority of these events were non-serious, mild, and unrelated to study drug.
- 4 patients experienced serious events, none of them were considered related to study drug.
- In general, most of the adverse events reported in this study were consistent with what would be expected in a pediatric Duchenne population.
- No serious hypersensitivity events were reported. Rash was the most frequently non-serious hypersensitivity event.
- No patients discontinued due to an adverse event.

1. ClinicalTrials.gov Identifier: NCT02310906. 2. Sarepta Therapeutics Data on File.

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A PROPRIETARY AND DIFFERENTIATED APPROACH IN RNA TECHNOLOGY

GENE THERAPY

PMO

PPMO

PHOSPHORODIAMIDATE MORPHOLINO OLIGOMERS (PMO) CHEMISTRY

Specificity: Enhanced affinity for targeting pre-mRNA for precise binding to RNA targets

Stability: Highly resistant to degradation by enzymes

Versatility: Ability to rapidly design and construct drug candidates that are specific for human or pathogen RNA; and target specific tissues

Safety: Built upon a charge-neutral backbone, which may be reflected in tolerability

SAREPTA THERAPEUTICS

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PEPTIDE PHOSPHORODIAMIDATE MORPHOLINO OLIGOMERS (PPMO) CHEMISTRY

Enhances PMO

- Same precision genetic medicine backbone
- Conjugated peptide greatly increases penetration
- Could potentially lead to more efficient dosing for patients

SAREPTA THERAPEUTICS

RESEARCH & DEVELOPMENT: HOW DOES IT WORK?

Proof-of-concept (Cell Culture)
Confirm drug's mechanism of action

Animal Studies (Mouse, Rat, NHPs)
Test drug safety
Test drug effect

Clinical Trial (Human)
Characterize safety
Smaller population

Clinical Trial (Human)
Characterize effect
Larger population

For more information on our pre-clinical work and Phase 1 study (5051-101, NCT03375255), see archived webinar with PPMD (9 May 2018) and archived presentation from the 2018 PPMD conference
https://www.parentprojectmd.org/may-9-webinar-introducing-ppmo-the-future-of-precision-rna-targeted-therapies-for-duchenne/

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AFTER A SINGLE DOSE OF PPMO, IMMUNOHISTOCHEMISTRY PROVIDED EVIDENCE OF DYSTROPHIN UPREGULATION AND PRODUCTION LOCALIZED AT THE SARCOLEMMA

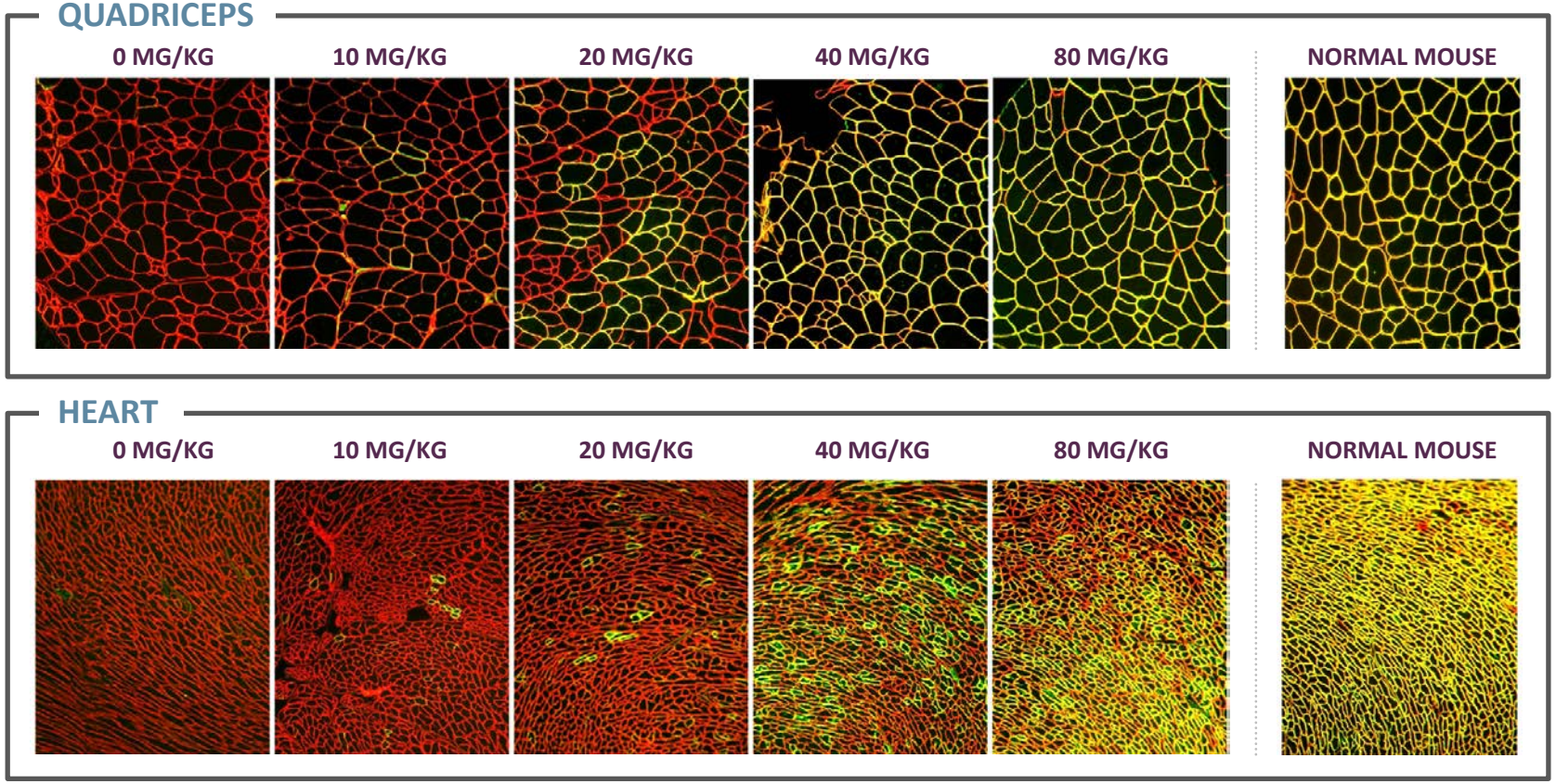
GENE THERAPY

PMO

PPMO

Double immunohisto-chemistry (Dystrophin/ Laminin)

INCREASING PPMO SINGLE DOSE



Yellow/orange/green: dystrophin-positive cells; Red: dystrophin-negative cells.
mdx mice at 7 weeks of age were treated with a single IV dose of PPMO at 10, 20, 40, or 80 mg/kg and analyzed at 30 days post-injection (N=4 mice per dose)
Experimental PPMO that targets exon 23 in *mdx* mouse.
Wu C, et al. Poster presented at: 2018 New Directions in Biology and Disease of Skeletal Muscle Conference. 25-28 June 2018. New Orleans, LA.

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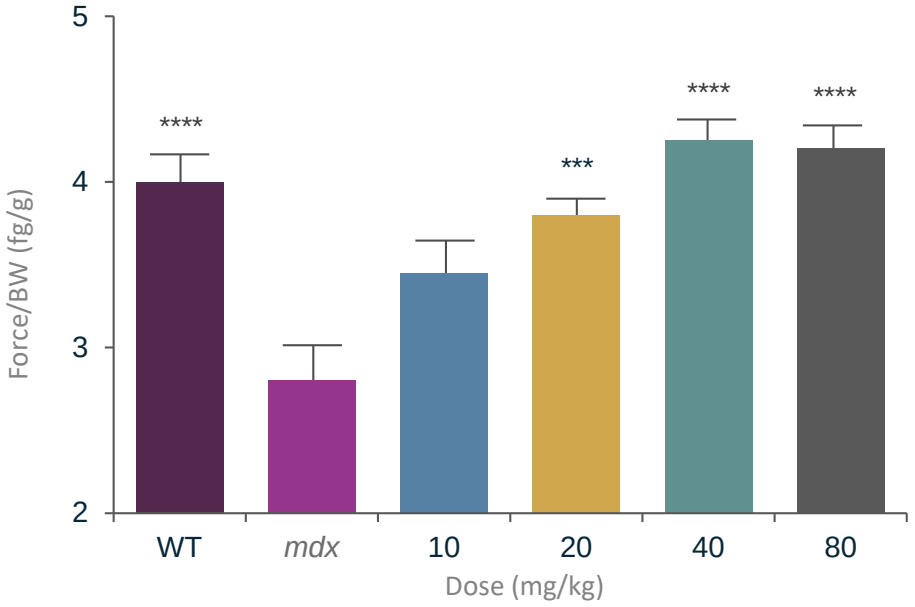
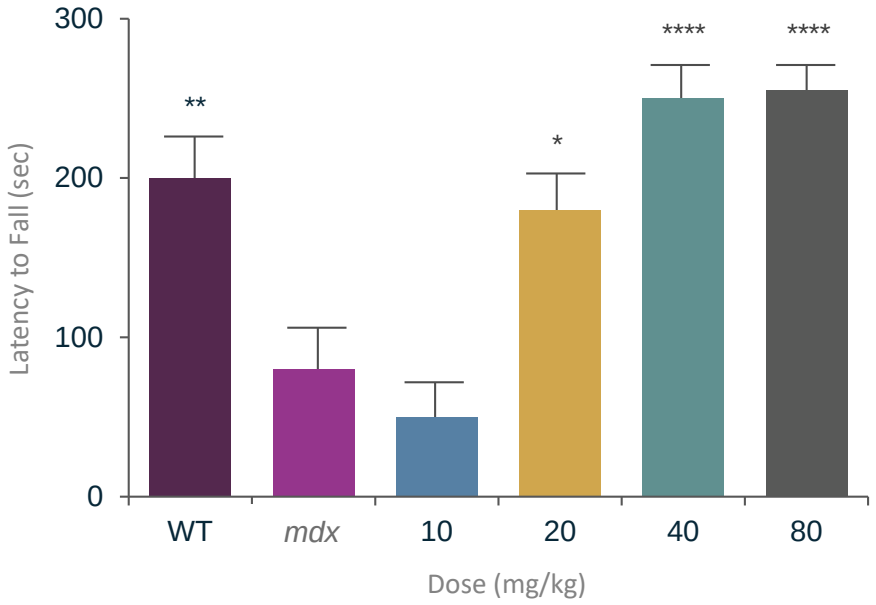
EFFECT OF PPMO TREATMENT ON MUSCLE FUNCTION IN 10-WEEK-OLD *MDX* MICE (DUCHENNE ANIMAL MODEL)

GENE THERAPY
PMO
PPMO

GRIP STRENGTH
(muscle strength)



ROTAROD
(muscle strength, coordination, and endurance)



BW, body weight; SE, standard error.

mdx mice at 7 weeks of age were treated with a single IV dose of saline or PPMO at 10, 20, 40, or 80 mg/kg, and WT mice at 7 weeks of age were treated with a single IV dose of saline. Mice were tested for grip strength at 10 weeks of age (3 weeks postinjection) and for rotarod at 9 weeks of age (2 weeks postinjection) (n=10 per group). Values shown are mean ± SE.

Statistics: One-way ANOVA Tukey multiple comparison test data and the significant values shown are vs *mdx* saline (*P<0.05, **P<0.01, ***P<0.001, ****P<0.0001).

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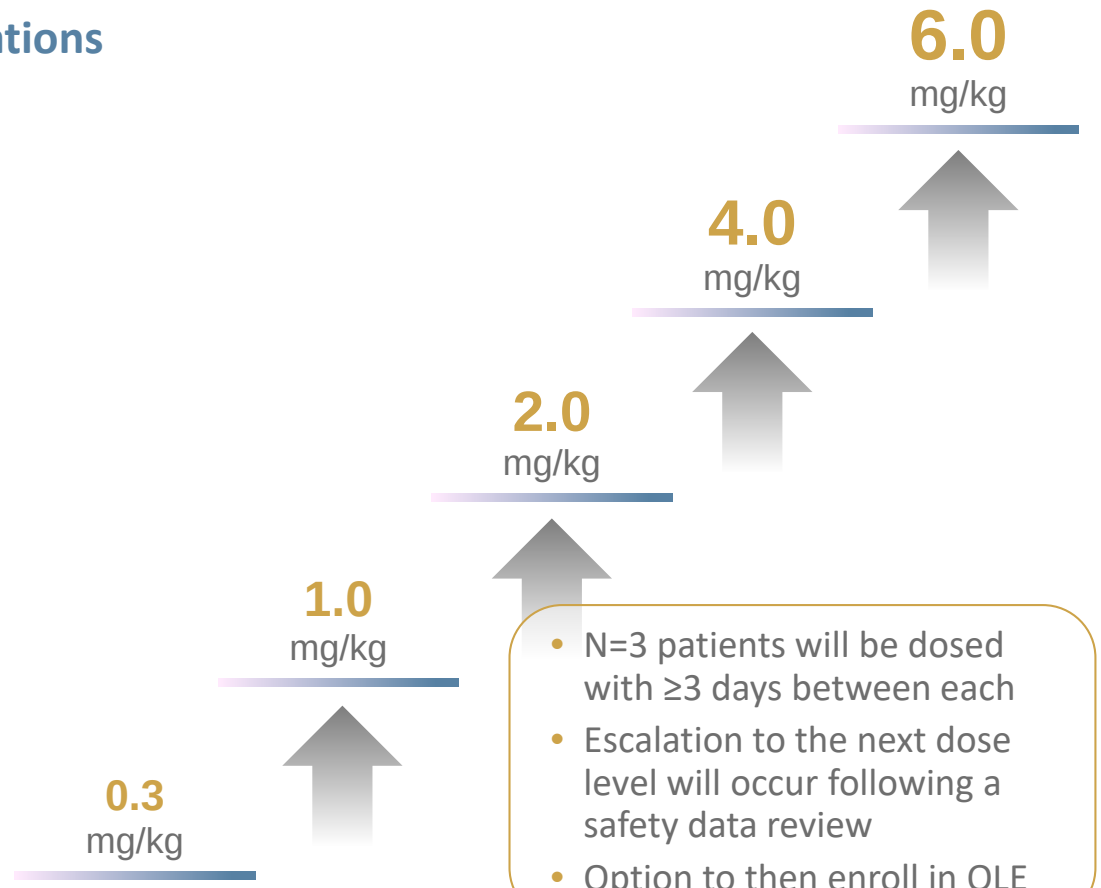
5051-101: PHASE 1 STUDY OVERVIEW (NCT03375255)

Single Ascending Dose (SAD) Study Evaluations

- Safety & tolerability
- Pharmacokinetics (PK)

Key Enrollment Criteria

- Patients age 12+ with DMD amenable to exon 51 skipping
- Stable dose of oral corticosteroids for 12 weeks OR no corticosteroids for 12 weeks prior to screening
- No exposure to exon-skipping agents for 6 months prior to screening
- No exposure to gene therapy
- Stable cardiac and pulmonary function
- No changes to cardiac medications for 12 weeks prior to screening



https://clinicaltrials.gov/ct2/show/NCT03375255 (accessed 08 Mar 2019), Sarepta Therapeutics Investigator Brochure. OLE, open-label extension.

SRP-5051 is investigational and not approved in the United States.

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5051-201 STUDY DESIGN CURRENTLY ENROLLING IN US AND SOON IN OTHER COUNTRIES

- A Phase 2, Two-Part, Multiple-Ascending-Dose Study of SRP-5051 for Dose Determination, then Dose Expansion, in Patients with Duchenne Muscular Dystrophy Amenable to Exon 51-Skipping Treatment
- Duchenne participants (ambulatory or non-ambulatory) amenable to Exon 51 skipping, ages 7 to 21 years, inclusive.
- Has been on a stable dose of oral corticosteroids for at least 12 weeks prior to study drug administration, or has not received corticosteroids for at least 12 weeks prior to study drug administration.
- Active sites in USA

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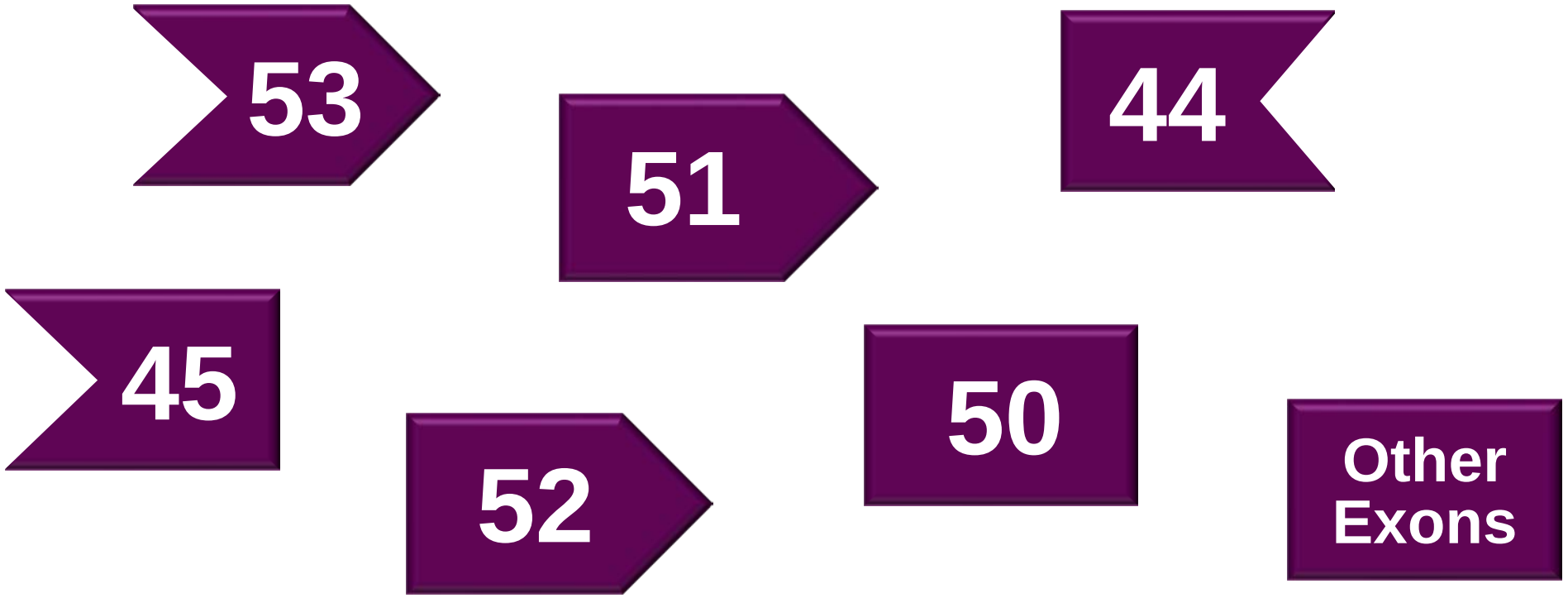
PPMO NEXT MUTATION



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THE SCIENCE WILL INFORM NEXT STEPS

In mid-2020 we plan to have more clarity around this decision.

FREQUENTLY ASKED QUESTIONS

1. *How do I decide between exon skipping and gene therapy for my family?*

2. *Will exon-skipping treatment impact my ability to receive gene therapy later?*

IN CLOSING

We have built an expansive pipeline dedicated to Duchenne muscular dystrophy with the goal of treating 100% of individuals with the disease.

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FINN
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