Our Vision For The Future of Precision Genetic Medicine in Duchenne
FORWARD-LOOKING STATEMENTS

This presentation contains forward-looking statements. These forward-looking statements generally can be identified by the use of words such as “believes” or “belief,” “anticipates,” “plans,” “expects,” “will,” “intends,” “potential,” “possible,” “advance” and similar expressions. These forward-looking statements include statements about Sarepta’s goals, strategy, pipeline, scientific approach, clinical development programs, plans regarding study designs, study sites, patient enrollment, the potential benefits of Sarepta’s product candidates and expected milestones, including the plan to file certain INDs.

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OUR URGENT MISSION

Our goal is to develop life-changing precision genetic medicine to treat 100% of individuals with Duchenne muscular dystrophy.

“I see other moms yell at their kids to stop running and I think how lucky they are to be able to do that.”*

- Katie, single mom with 6-year-old son diagnosed with Duchenne at age 2

Archie wants a cure. Isaac wants new legs so he can run faster like his friends, and George wants a Jacuzzi to help his muscles.”

- Paula, mom with 3 boys (age 11, 6, and 6) diagnosed with Duchenne, talking about her son’s letters to Santa Claus

*Granted Permission to Use
THE BUILDING BLOCKS FOR OUR STRATEGY

- Transforming pipeline and accelerating R&D efforts
- Resources to fuel future growth
- Bolstering infrastructure and manufacturing capabilities
- Attracting and retaining top talent
INTRODUCING NEW SENIOR LEADERSHIP

Louise Rodino-Klapac, Ph.D
Head of Gene Therapy

Chief Medical Officer
DIVERSE PRECISION GENETIC MEDICINE PIPELINE
16 DUCHENNE PROGRAMS CURRENTLY IN DEVELOPMENT

- RNA-targeted Therapies
- Gene Therapy
- Gene Editing
- Additional Approaches

11 PROGRAMS
5 MUTATION AGNOSTIC DUCHENNE PROGRAMS
OUR SCIENTIFIC APPROACH: RNA-TARGETED THERAPIES

- Exon-skipping is a potential treatment approach to correct for specific genetic mutations and restore production of shortened, but functional, dystrophin protein.
- We are rapidly advancing multiple exon-skipping therapies, with the goal of treating all individuals with Duchenne amenable to this approach.
PMO: OUR CORE EXON-SKIPPING TECHNOLOGY

ENABLES THE DEVELOPMENT OF NEW THERAPIES

Precision

PMOs are engineered to bind sequence-specifically to RNA targets with precision

Safety

PMOs have a well-characterized and established safety profile in clinical testing and real-world use
PPMO: PMO with the addition of a cell-penetrating peptide, potentially leading to:
— Higher levels of dystrophin production
— More efficient dosing for patients
PPMO PRE-CLINICAL DATA
SRP-5051 & SRP-5053
GLOBAL DELIVERY OF SRP-5051 (PPMO-51) TO ALL RELEVANT MUSCLE GROUPS IN NON-HUMAN PRIMATES (PRECLINICAL DATA)

- SRP-5051 = Exon-51 PMO + peptide
- Widespread and high levels of exon skipping were observed in muscles that are highly affected in Duchenne, including >90% exon skipping in quadriceps and diaphragm and >60% in heart and smooth muscles

Internally generated NHP data.
PPMO IMPROVES MUSCLE FUNCTION IN PRECLINICAL STUDIES

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 post-injection) and for rotarod at 9 weeks of age (2 weeks post-injection) (n=10 per group). Values shown are mean ± SE. Statistics: One-way ANOVA Tukey multiple comparison test and the significant values shown are vs mdx saline (*P<0.05, **P<0.01, ***P<0.001, ****P<0.0001).
PPMO 5051-101 PHASE 1 STUDY (NCT03375255): PARTICIPANT EXPERIENCE/COMMITMENT

- Participants will receive 1 dose of PPMO, and will visit their study site 5x in ~14 weeks
- All participants who complete the study will have the opportunity to enroll in an open-label extension study (5051-102)

Learn more by watching our PPMO Webinar with PPMD:
Visit the PPMD website (News: May 31 2018) for a link to the audio recording and the presentation.
SRP-5053 ALSO PROMISING IN PRECLINICAL STUDIES (NHP)

SRP-5053 = Exon-53 PMO + peptide

Internally generated NHP data.
We aim to file an IND for PPMO SRP-5053 in late 2018, with additional plans to file INDs in 2019.

Currently enrolling Phase 1 clinical trial with open-label extension study.

Pending results of early clinical studies.
Sarepta Therapeutics is currently developing three gene therapies for the treatment of Duchenne, representing two distinct therapeutic approaches, and is partnered with luminaries in this field of research.

**Pioneers in gene therapy**

*Working on two gene therapies developed and led by Jerry Mendell, M.D., Louise Rodino-Klapac, Ph.D., and Kevin Flanigan, M.D.*

**A premier industry partner**

*First organization to demonstrate potential for micro-dystrophin gene therapy to restore muscle function in large animal study*
The Viral Vector

- Our vector was isolated from a rhesus monkey, not derived from a human host
- This, and our ongoing studies, suggest low pre-existing immunogenicity (still under evaluation, currently <15%)¹
- Pre-clinical work demonstrates delivery to skeletal, diaphragm & cardiac muscle, without crossing blood brain barrier

The Promoter

- In preclinical studies, MHCK7 promoter enables widespread micro-dystrophin expression across all muscle types

Our Micro-Dystrophin Gene

- Genetic engineering of micro-dystrophin gene based upon clinical observation²
- Maintains spectral-like repeats 1, 2, 3, and 24 (SR1, SR2, SR3, and SR24)

¹ Unpublished data presented by Dr. Louise Rodino-Klapac at Sarepta’s 2018 R&D Day on June 19, 2018 in New York City.
ADAPTIVE CLINICAL STUDY APPROACH: COHORT C (4-7 YEARS): ADDITION OF PLACEBO-CONTROLLED STUDY COHORT

Subjects
- Treatment arm, n=12
- Placebo arm, n=12
  - Crossover at 1 year

Endpoints
- Primary:
  - Safety
  - Demonstration of micro-dystrophin protein expression
- Secondary:
  - Decrease in CK
  - Time to rise
  - Ascend 4 steps
  - NSAA
  - 10-meter timed test
  - 100-meter timed test
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Join our mailing list to get the latest updates on our progress:
duchenne.com/connect
THANK YOU

Special thanks to the patients and families who have participated in our clinical trials and to our many partners in this important work.