SAREPTA
THERAPEUTICS

CREDIT SUISSE HEALTHCARE CONFERENCE
SCOTTSDALE, ARIZONA

November 7, 2017

NASDAQ: SRPT

FORWARD-LOOKING STATEMENTS

This presentation contains "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Statements that are not historical facts or words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," “goal,” ”strategy,” ”may,” “should,” “project,” “estimate,” and similar expressions are intended to identify forward-looking statements. Forward-looking statements in this presentation include but are not limited to: Sarepta being the leader in precision genetic medicines for rare neuromuscular diseases, commencing with DMD; Sarepta leading the way in DMD treatment, expanding globally and advancing its multiplatform approach to treating rare NMD; Sarepta’s goal to help patients around the world; Sarepta’s robust pipeline and technology, including in collaboration with third parties, and their respective potential benefits and expected milestones for the same; Sarepta’s revenue guidance of $150 to $155M for the year 2017; the potential for PMO and PPMO to impact the goal of Sarepta’s PMO technology to improve outcomes for children with DMD through delaying the loss of ambulation and pulmonary function, and through greater function; the goal of Sarepta’s PPMO platform to transform the lives of DMD children; PPMO being a potential transformative approach to slowing disease progression in DMD and holding the promise of transforming standard-of-care; the potential of PMO to bring DMD kids closer to an Exon 44 phenotype and PPMO to bring DMD kids closer to non-DMD phenotype and other potential benefits of PMO and PPMO, the initiation of a SAD study and its design; and other statements made during the presentation regarding Sarepta’s future, strategy and business plans.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta’s control and are based on Sarepta’s current beliefs, expectations and assumptions regarding it 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 Sarepta’s business, results of operations and trading price. Potential known risk factors include, among others, the following: we may not be able to meet expectations with respect to EXONDYS 51 sales or attain the net revenues we anticipate for 2017, profitability or positive cash-flow from operations; we may not be able to comply with all FDA post-approval commitments/requirements with respect to EXONDYS 51 in a timely manner or at all; we may not be able to obtain regulatory approval for eteplirsen in jurisdictions outside of the U.S. including from the EMA; we may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of any of our product candidates, including PPMO; the results of our ongoing research and development efforts, including those with strategic partners, and clinical trials for our product candidates, including PPMO, may not be positive or consistent with prior results or demonstrate a safe treatment benefit which could negatively impact our business; we may not be able to execute on our business plans, 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 with respect to patents that cover our product candidates; and those risks identified under the heading “Risk Factors” in Sarepta’s 2016 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2017 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.

CORPORATE PROFILE

*Sarepta Therapeutics is a **leader in precision genetic medicines** for rare neuromuscular* diseases, ***commencing with Duchenne muscular dystrophy (DMD).***

STRATEGIC IMPERATIVES

Leading the way in DMD treatment

EXONDYS 51® (eteplirsen) injection is the first precision medicine approved in the U.S. to treat the underlying cause of DMD

Expanding globally

Our goal is to help patients around the world

Advancing our multi-platform approach to treating rare NMD

Investing in our robust pipeline:

- *Our exon skipping platform*
- *Our next generation RNA platform*
- *Our precision gene therapy and gene editing collaborations*

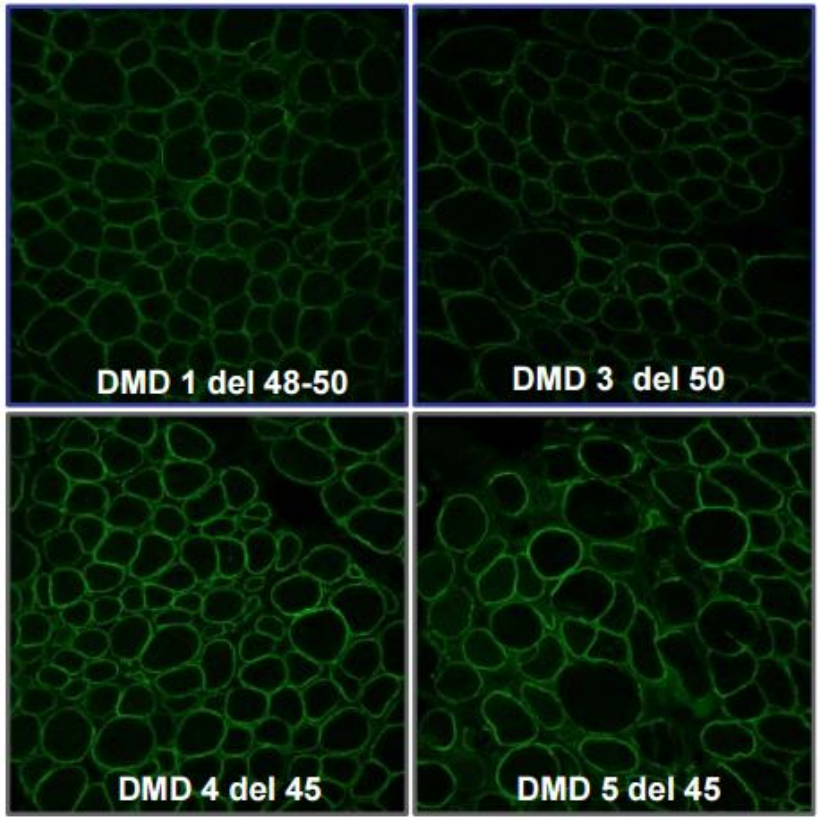
EXONDYS 51®: THE FIRST APPROVED THERAPY IN U.S. FOR DMD

POTENTIAL TO TREAT 13 PERCENT OF THE DMD POPULATION

- ✓ Granted accelerated approval by the U.S. Food and Drug Administration in September 2016 on the basis of compelling evidence that dystrophin production is reasonably likely to predict a clinical benefit
- ✓ Indicated for the treatment of DMD in patients amenable to exon 51 skipping
- ✓ Targets dystrophin deficiency, the underlying cause of DMD
- ✓ Post-marketing confirmatory trial requirement

THE IMPORTANCE OF DYSTROPHIN IN DMD

DMD PATIENTS WITH A DELETION OF EXON 45 HAVE SPORADIC EXON-SKIPPING ASSOCIATED WITH SLOWER DISEASE PROGRESSION



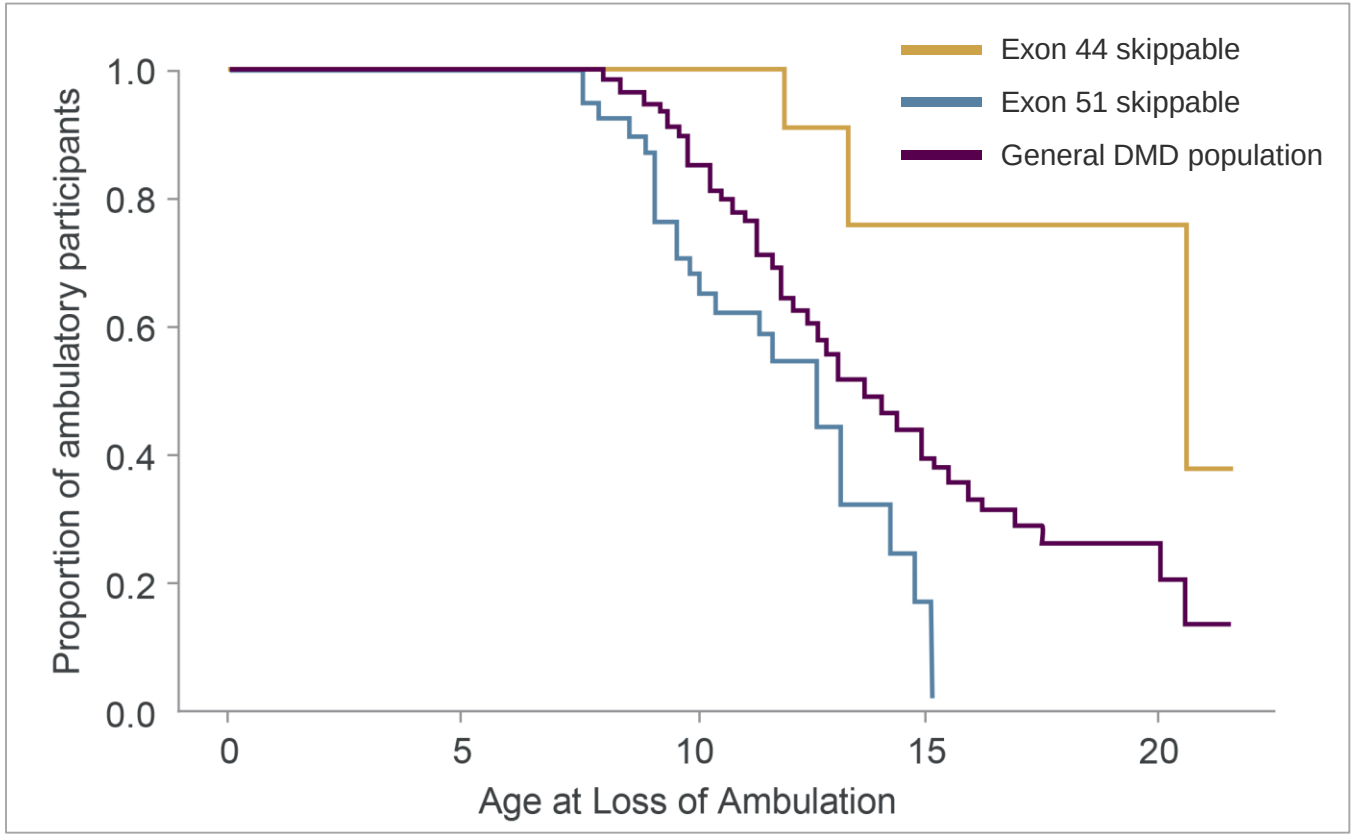
ENDOGENOUS AND SPORADIC EXON-SKIPPING OF EXON 44 LEADS TO:

1. Detectable exon-skipping (RT-PCR) and very low level of increased baseline dystrophin (occasionally below level of detection)
2. Significantly higher baseline function
3. Significantly longer period of ambulation
4. Significantly lower rate of disease progression

Source Beekman C, Sipkens JA, Testerink J, Giannakopoulos S, Kreuger D, et al. (2014) A Sensitive, Reproducible and Objective Immunofluorescence Analysis Method of Dystrophin in Individual Fibers in Samples from Patients with Duchenne Muscular Dystrophy. PLoS ONE 9(9): e107494

PATIENTS WITH DELETIONS OF EXON 45 HAVE DELAYED LOSS OF AMBULATION COMPARED TO THE GENERAL DMD POPULATION

EXON 44 AND 51 SKIPPABLE MUTATIONS



Ambulation >14 years old by mutation type

71.4% exon 44 skippable

12.5% exon 51 skippable

Source Moon, D.; Bange, J.; Horn, P.; Rybalsky, I.; Shellenbarger, K.C., Tian C.; Wong, BL. Genotype-Phenotype Associations in a Large Cohort of Duchenne Muscular Dystrophy Patients. 22nd International Congress of the World Muscle Society; October 3-7, 2017; St. Malo, France.

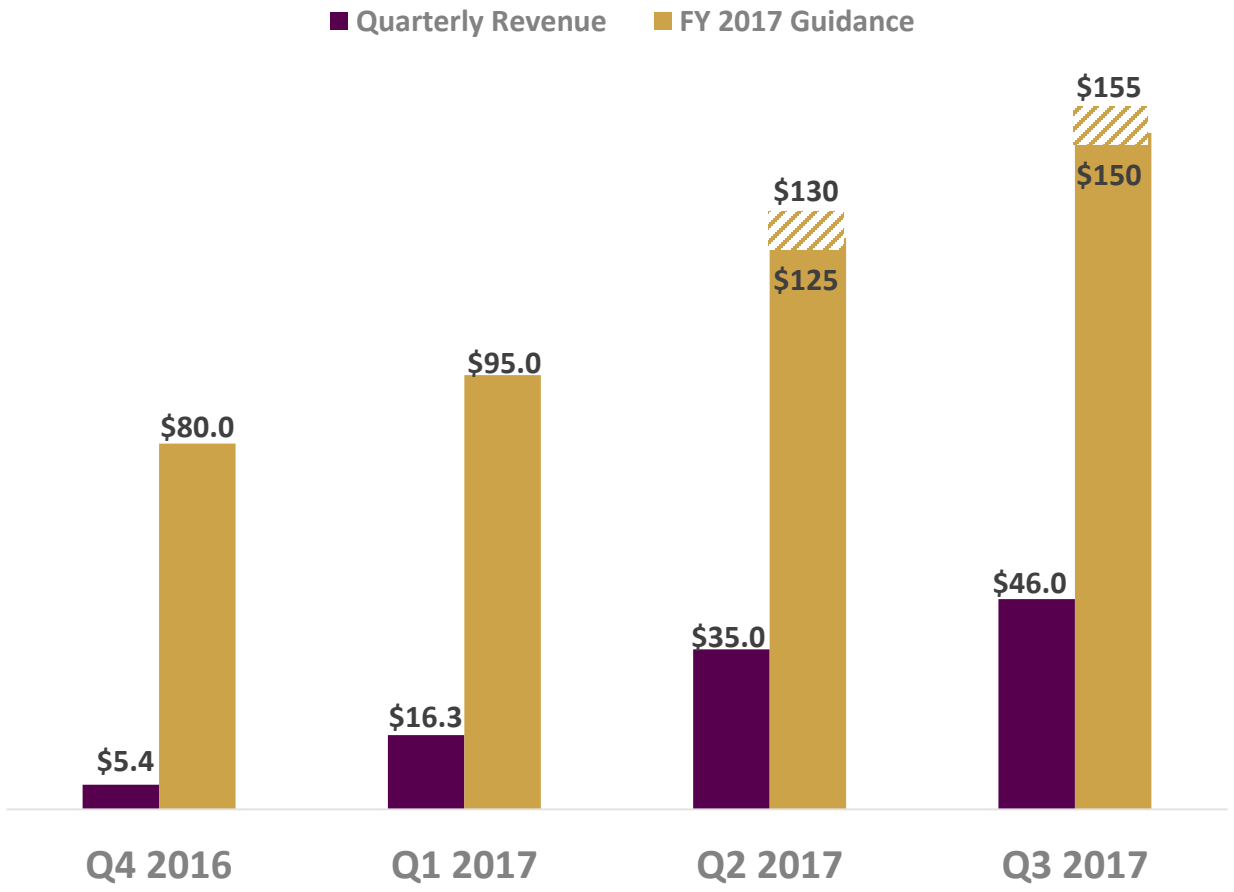
FINANCIAL HIGHLIGHTS

Successful launch of EXONDYS 51 with over 160 physicians submitting a START Form

Q3 2017 revenue of $46M versus $35M in Q2 2017

Raised full-year 2017 revenue guidance to $150 to $155M from $125 to $130M

$618M in cash equivalents, restricted cash and investments at September 30, 2017



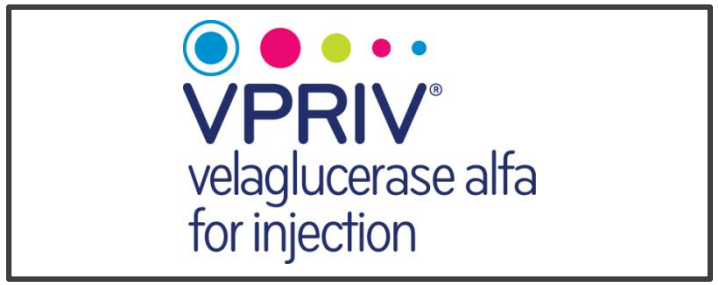
EXONDYS 51 RANKS AMONG THE TOP FIVE U.S. RARE DISEASE DRUG LAUNCHES IN HISTORY*

Sarepta Therapeutics

Vertex

Vertex

Biogen



Shire

based on projected first full-year U.S. sales

PIPELINE PROGRESS:
- PPMO IND
- MICRO-DYSTROPHIN IND
- GALGT2 IND

OUR PRECISION GENETIC MEDICINE TECHNOLOGY

PHOSPHORODIAMIDATE MORPHOLINO OLIGOMER (PMO)

PMO platform is precision genetic medicine

- PMO induces proper exon-skipping and creates in-frame RNA transcript (shown 100 percent of the time in patients across PMO candidates)
- Transcript produces dystrophin as measured by Western blot
- Dystrophin properly localizes to the sarcolemma, indicating that it is functional

PMO performance

- Dystrophin production is about 1 percent of normal at 2.5 years for EXONDYS 51 and about 1.02 percent at 1 year for golodirsen, a more efficient sequence

Exon 44 dystrophin production

- Exon 44 patients have sporadic endogenous exon-skipping
- Trace dystrophin: Often below detectable levels on Western Blot

Exon 44 phenotype

- Ambulatory five years longer than other DMD populations
- Significant improvement on 6MWT and in change to 6MWT over time

Goal of our PMO technology is to improve outcomes for children with DMD:

- Delay loss of ambulation
- Delay loss of pulmonary function
- Greater function

Typical DMD

Phenotype

- Out of frame deletion
- Dystrophin: Usually undetectable
- Loss of ambulation: 11-13
- Mortality: Teens to 20s

Exon 44 Skippable

Phenotype

- Sporadic background skipping
- Dystrophin: Exon-skipping detected, but dystrophin often below detectable levels
- Loss of ambulation: >15

Becker's Patient

Phenotype

- Inframe deletion
- Dystrophin: Usually 3 percent or higher
- Loss of ambulation: Heterogeneous, but often 20+ to full life

Non-DMD

- Pompe disease
- Myotonic dystrophy
- Facioscapulohumeral muscular dystrophy
- Friedreich's ataxia
- Progeria

PMO

OUR NEXT-GENERATION PRECISION GENETIC MEDICINE TECHNOLOGY

PEPTIDE PHOSPHORODIAMIDATE MORPHOLINO OLIGOMER (PPMO) CHEMISTRY

PPMO enhances PMO

- Same precision genetic medicine backbone
- Conjugated peptide greatly increases penetration
- Transcript produces dystrophin as measured by Western blot
- Dystrophin properly localizes to the sarcolemma, indicating that it is functional

Animal models show step order increase in efficacy

- 10-30X increase in dystrophin production in *mdx* mouse model
- Dystrophin production in skeletal, smooth, and cardiac muscle

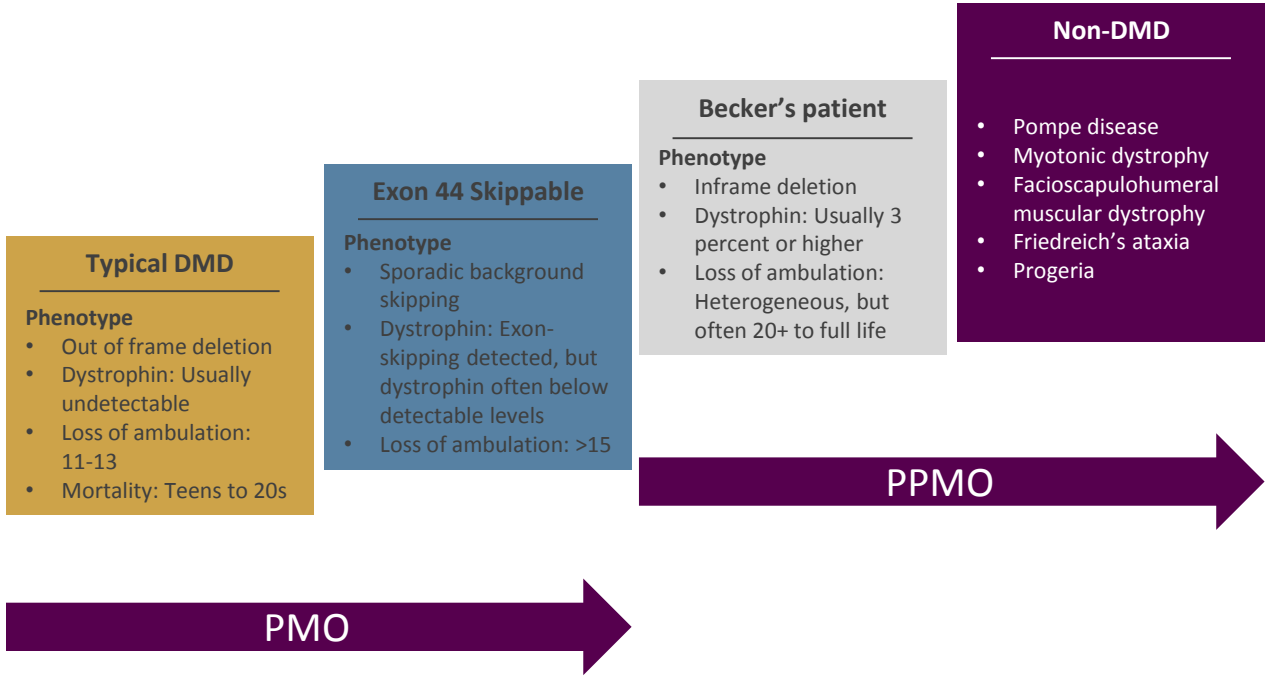
PPMO holds the promise of transforming standard-of-care

- Becker's patients have 3 percent or greater dystrophin production
- Loss of ambulation greater than 20 years to full life
- Some Becker's patients live into their 60s

Goal of our PMO technology is to improve outcomes for children with DMD:

- Delay loss of ambulation
- Delay loss of pulmonary function
- Greater function

Goal of our PPMO platform is to transform the lives of DMD children



PPMO OVERVIEW

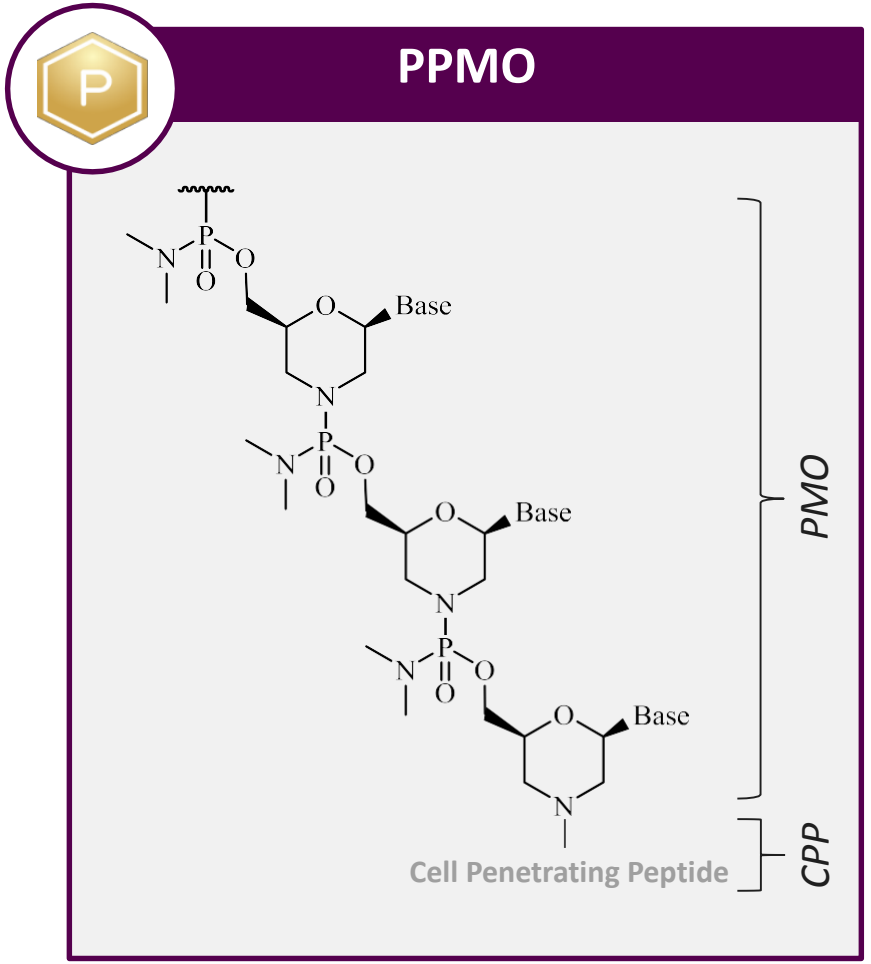
DESIGNED TO ENHANCE TISSUE TARGETING, INTRACELLULAR DELIVERY, SELECTIVITY AND POTENCY

Proprietary class of PPMO compounds have the potential to provide:

- Improved delivery *in vivo*
- Superior dystrophin production *in vivo*
- Tolerability in non-human primates
- Less frequent dosing
- Longer patent term

PPMO has the potential to address multiple neuromuscular diseases, including:

- Pompe disease
- Myotonic dystrophy
- Facioscapulohumeral muscular dystrophy
- Friedreich's ataxia
- Progeria

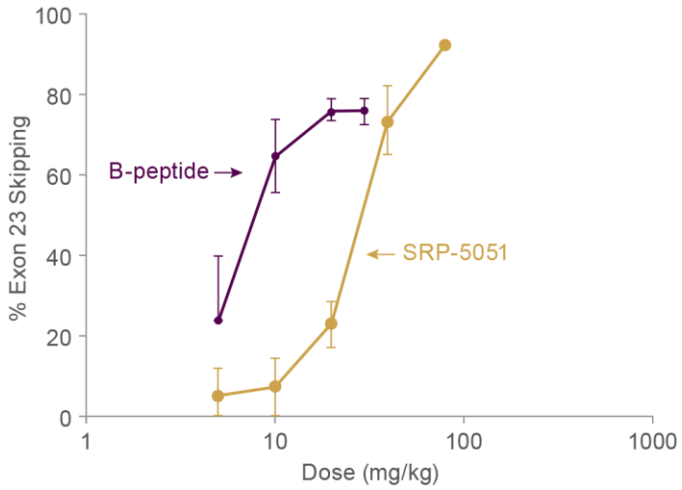


SRP-5051: A VIABLE CLINICAL CANDIDATE

LEARNING FROM PAST EXPERIENCES; SAFETY NO LONGER A RATE-LIMITING FACTOR TO CLINICAL DEVELOPMENT

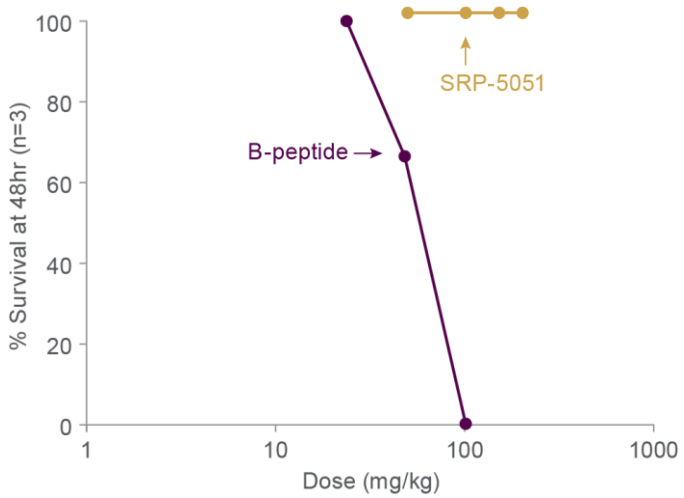
HIGH EFFICACY

Exon Skipping in Quadriceps

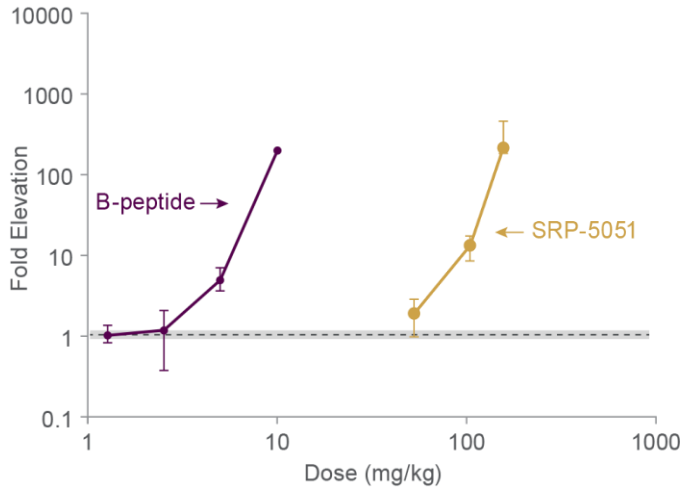


IMPROVED TOLERABILITY

Survival

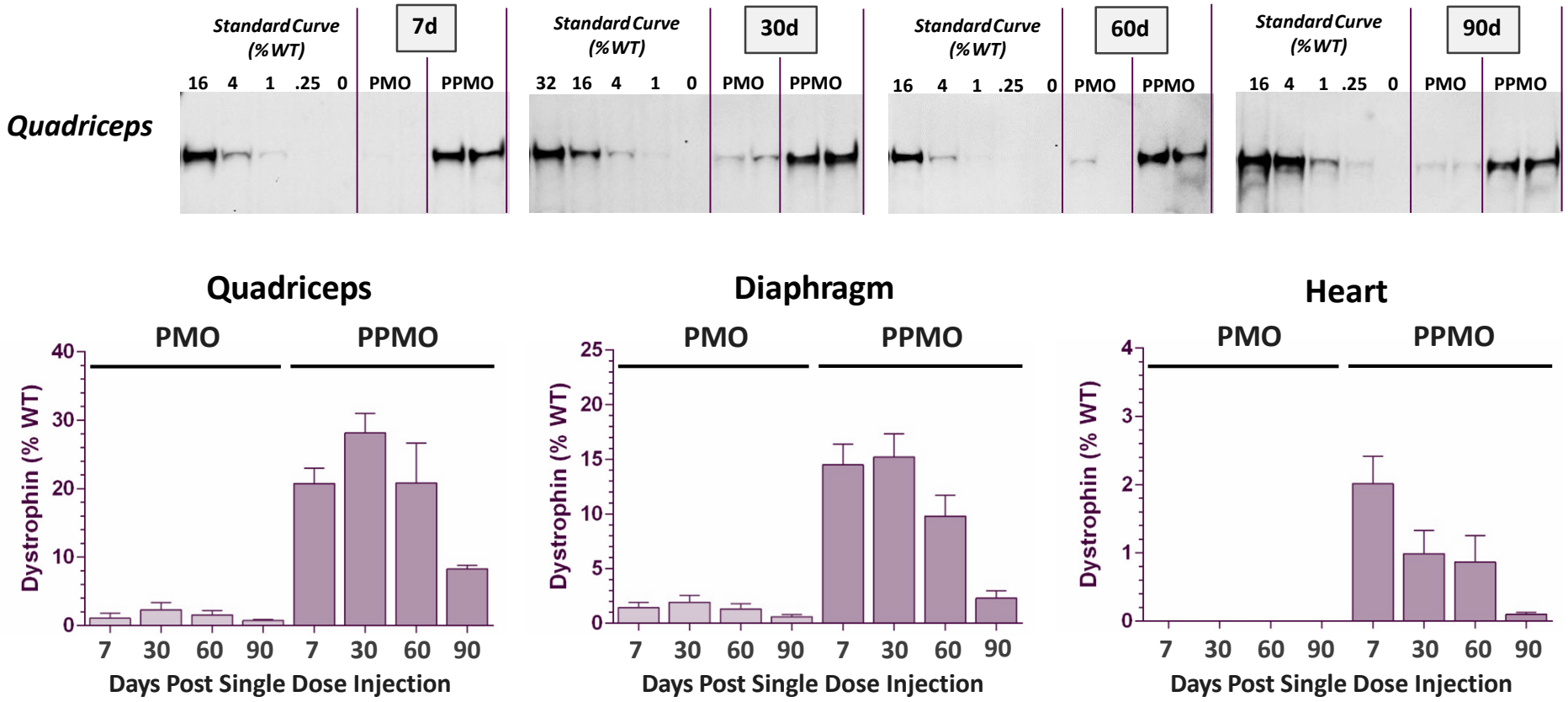


Induction of KIM-1



A SINGLE PPMO DOSE SUSTAINS INCREASED LEVELS OF DYSTROPHIN FOR 90 DAYS IN *MDX* MICE

RESPONSE DURABILITY SUPPORTS LESS FREQUENT DOSING

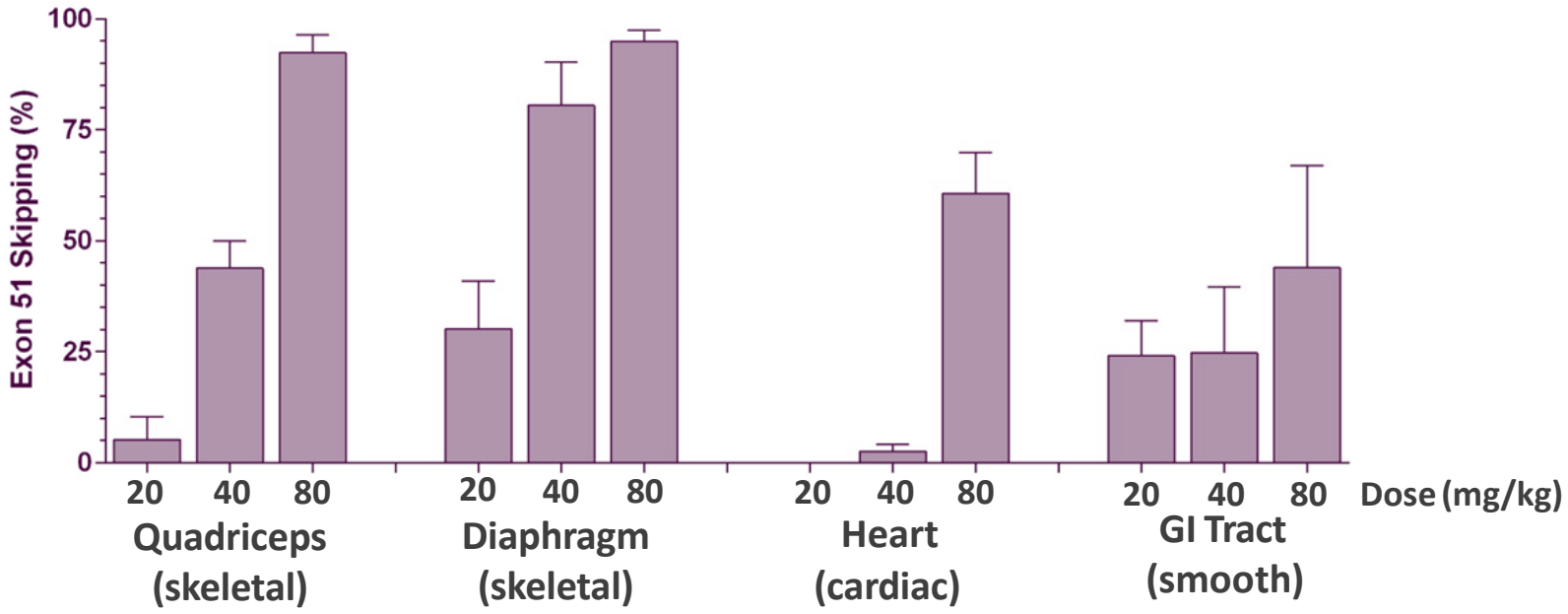


MDX (DMD) mice were treated with a single IV dose of PMO or PPMO @ 40 mg/kg

The clinical Western blot method for dystrophin was performed on muscle at 7-90 days post single dose injection

SRP-5051 ACHIEVES DELIVERY IN ALL RELEVANT MUSCLE GROUPS

GREATER THAN 90 PERCENT EXON 51 SKIPPING IN NON-HUMAN PRIMATE (NHP) STUDY



Exon-skipping observed in all relevant muscle groups: skeletal, cardiac and smooth muscle

NHPs tolerated 4-weekly doses of 20, 40 and 80 mg/kg

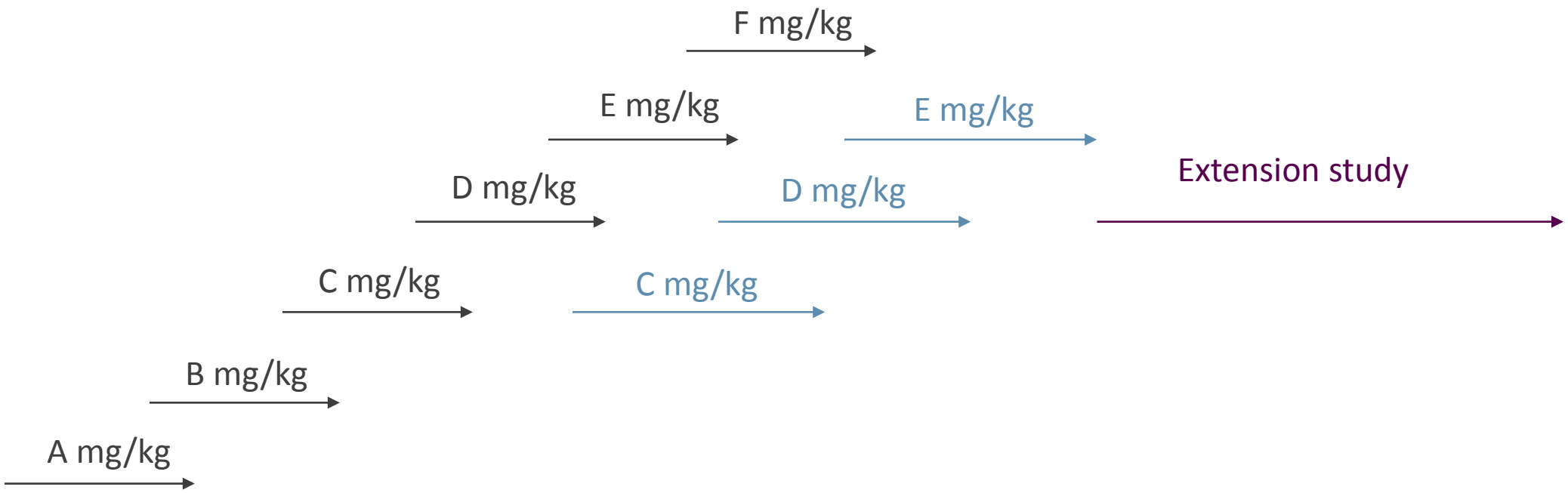
INITIATING CLINICAL TRIAL FOR SRP-5051

SAD/MAD COMBINATION STUDY FOLLOWED BY AN EXTENSION STUDY

Initiate single-ascending dose (SAD) study at appropriately conservative levels

Use PK/PD modeling to confirm assumptions regarding dose escalation and guide the start of an overlapping multiple-ascending dose (MAD) study to identify therapeutic dose

Initiate an extension study to accrue further safety experience and provide more evidence regarding the effects on dystrophin



PPMO: A POTENTIALLY TRANSFORMATIVE APPROACH TO SLOWING DISEASE PROGRESSION IN DMD

- PPMO is a superior delivery vehicle for muscle penetration compared to PMO
- PPMO induces significant increases in dystrophin levels that is sustained for 90 days
- Long duration of action may correlate with less frequent dosing in the clinic
- SRP-5051 targets the three major muscle groups affected in DMD (skeletal, cardiac, smooth)
- Widespread exon-skipping in NHPs indicates that the therapeutic effect is scalable to large mammals
- Pre-clinical data strongly support clinical development of SRP-5051

EXTENDING PRECISION GENETIC MEDICINE THROUGH EXTERNAL PARTNERSHIPS

LEADING THE WAY IN GENE THERAPY FOR DMD

POTENTIAL TO ADDRESS ALL PATIENTS WITH DMD, COMPLEMENTING EXON-SKIPPING APPROACHES

PARTNER	Nationwide Children's Hospital	Nationwide Children's Hospital	Genethon	Duke University
ANNOUNCED	January 2017	January 2017	June 2017	October 2017
MODALITY	Micro-Dystrophin Gene Therapy	GALGT2 Gene Therapy	Micro-Dystrophin Gene Therapy	CRISPR/Cas9
TYPE	Research and option agreement	License agreement	Research and option agreement	Research and option agreement
STATUS	IND Accepted	IND Accepted	Manufacturing scale-up underway	Technology optimization

THANK YOU