



SAREPTA
THERAPEUTICS

INVESTOR PRESENTATION

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, with a primary focus on DMD; 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; the potential of PPMO and the plan to file IND in 2017 and to initiate a study by year-end of 2017; the benefits and rights of the BioMarin settlement and license agreements, including the agreements providing Sarepta with worldwide freedom to operate for EXONDYS 51 and future exon-skipping products, and the payments and royalties that Sarepta will be making as part of these agreements; Sarepta's strategic plans, including its plans to continue the positive commercial launch trajectory, to expand its EU footprint and build infrastructure by 2018, to expand its Managed Access Program ("MAP") and to realize revenue from its MAP in Q4 2017, and specific plans regarding pipeline advancement; Sarepta's planned clinical studies, including in connection with FDA post-marketing commitment/requirements, and expected milestones, timelines and design; the data Sarepta submitted to the European Medicines Agency ("EMA") supporting the clinical efficacy of EXONDYS 51 and expectations relating to the marketing authorization application evaluation process and timelines; the results of Golodirsen (SRP-4053) further validating Sarepta's exon-skipping platform for the treatment of DMD, 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 its 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; the results of our ongoing research and development efforts, including those with strategic partners, and clinical trials for our product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit which could negatively impact our business; our rights to commercialize EXONDYS 51 and our follow-on exons across the world may not be fully protected by our patents and/or third party agreements; the expected benefits and opportunities related to the settlement and license agreements with BioMarin may not be realized or may take longer to realize than expected; we may not be able to establish and successfully conduct a MAP in one or more countries, and even if such program(s) are successfully conducted in each country targeted, we may not achieve any significant revenues from sales of eteplirsen under these programs; we may not be able to expand our MAP to other countries; 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 June 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.

GENERAL CORPORATE OVERVIEW



CORPORATE PROFILE

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

EXECUTIVE MANAGEMENT



Douglas S. Ingram, J.D.
President and Chief Executive Officer



Sandy Mahatme
*Executive Vice President
Chief Financial Officer and Chief Business Officer*



Catherine Stehman-Breen, M.D., M.S.
*Senior Vice President
Chief Medical Officer*



Guriqbal S. Basi, Ph.D.
*Senior Vice President
Chief Scientific Officer*



Bo Cumbo
*Senior Vice President
Chief Commercial Officer*



Shamim Ruff
*Senior Vice President
Regulatory Affairs and Quality*



Ty Howton
*Senior Vice President
General Counsel and Corporate Secretary*

GLOBAL FOOTPRINT



CAMBRIDGE, MASS.

In 2013, Sarepta established its U.S. corporate headquarters in a premier life sciences hub to tap into Massachusetts' talented and highly-educated workforce.



ANDOVER, MASS.

Sarepta's state-of-the-art research and manufacturing facility opened in a nearby suburb of Boston to significantly enhance the Company's in-house discovery and pre-clinical manufacturing capabilities.



ZUG, SWITZERLAND

In 2017, Sarepta established its European headquarters in Zug, a centrally located life science cluster, to support its global expansion efforts.

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
proximate causes of DMD*



Expanding globally

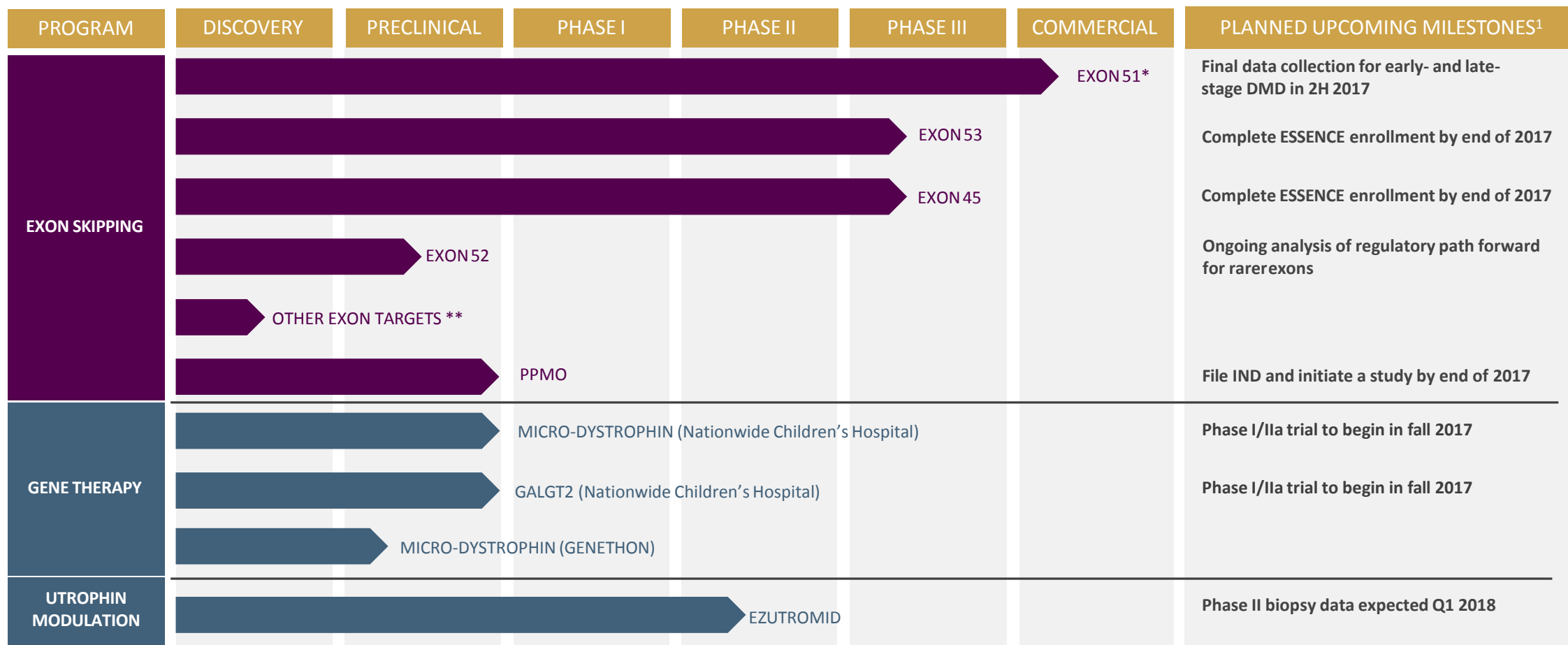
*Our goal is to help patients
around the world*



Staging a multi-front battle against DMD

*Robust pipeline comprising
follow-on exon-skipping therapies
and strategic partnerships for
new modalities*

DEEP PIPELINE



■ Internal
■ External Collaborations

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

**Other exon targets in development: 8, 35, 43, 44, 50, and 55

¹Each of the below is based on our current expectations, but any of which could be delayed or modified for reasons that may or may not be within our control

INVESTMENT RATIONALE



Year-to-date accomplishments

Commercial updates

- ✓ Raised revenue guidance range to \$125 – \$130 million for 2017
- ✓ Increased genetic testing and interpretation
- ✓ Continued KOL engagement/education

Global expansion

- ✓ Settled IP litigation; freedom to operate
- ✓ Established EU headquarters
- ✓ Launched Managed Access Program

Pipeline advancement

- ✓ Signed multiple collaborations, bolstering pipeline
- ✓ Announced positive results of golodirsen (SRP-4053) in patients with DMD amenable to skipping exon 53
- ✓ Presented data at MDA conference
- ✓ Appointed chief medical and scientific officers



Potential near-term catalysts

Commercial updates

- Continue positive launch trajectory
- EMA review is anticipated to be completed in 1H 2018

Global expansion

- Expand EU footprint; build infrastructure by 2018
- Realize revenue from Managed Access Program

Pipeline advancement

- Publish pulmonary data in peer-reviewed journal
- Define post-marketing commitment
- Commence PPMO clinical trial by end of 2017
- Commence two clinical trials in gene therapy by fall 2017
- Complete ESSENCE trial enrollment (exons 45/53) by end of 2017
- Announce PhaseOUT DMD Phase 2 data in Q1 2018

LEADING THE WAY IN DMD
TREATMENT



WHAT IS DMD?¹⁻²

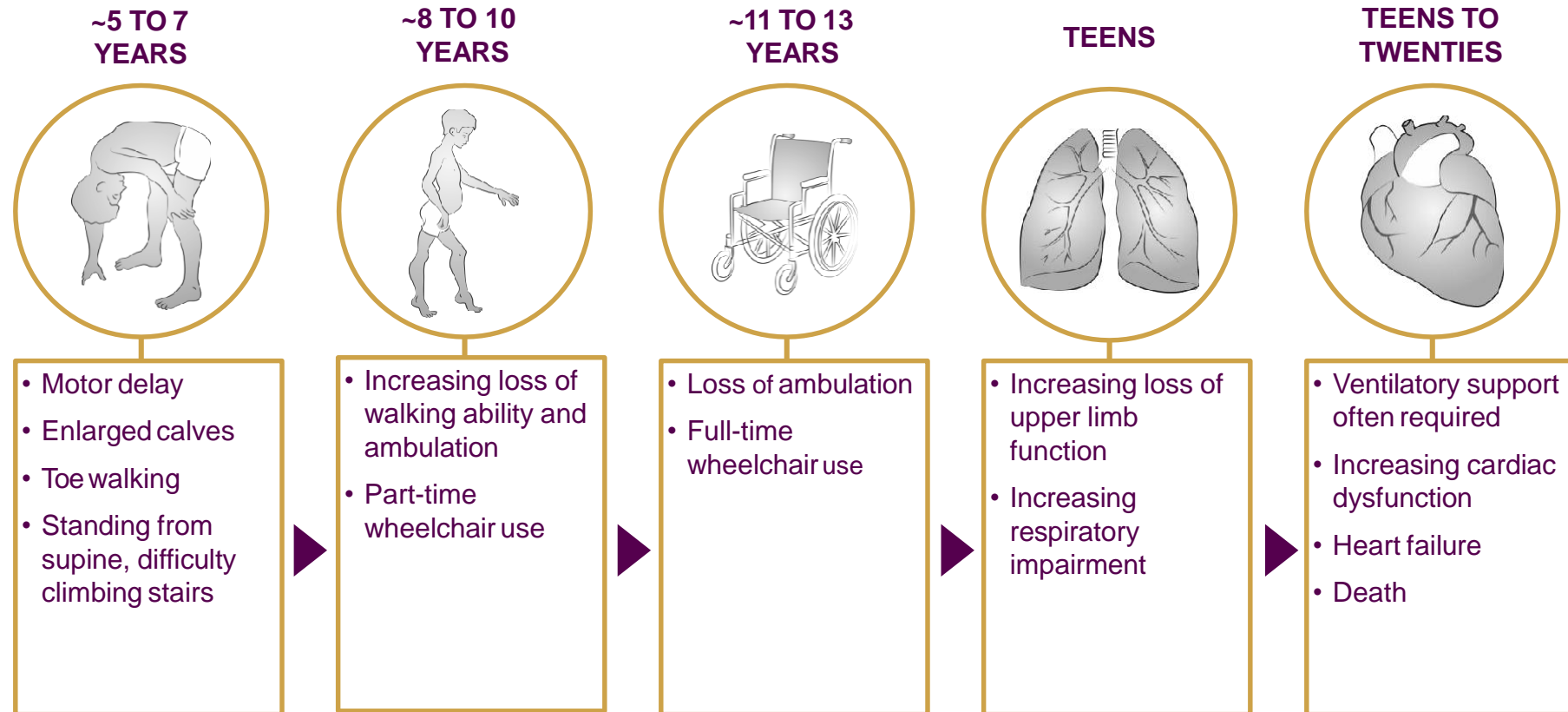
AFFECTS 1 IN 3,500-5,000 MALES BORN WORLDWIDE



- Rare, fatal progressive neuromuscular genetic disease
- Average lifespan of mid- to late-20s; typical diagnosis occurs between ages 4-5
- Caused by gene mutation that encodes dystrophin, a protein that plays a key structural role in muscle fiber production

1. Emery AE, Population frequencies of inherited neuromuscular diseases—a world survey. *Neuromuscul Disord.* 1991;1(1):19–29pmid
2. Emery AE. The muscular dystrophies. *BMJ.* 1998;317(7164):991-995

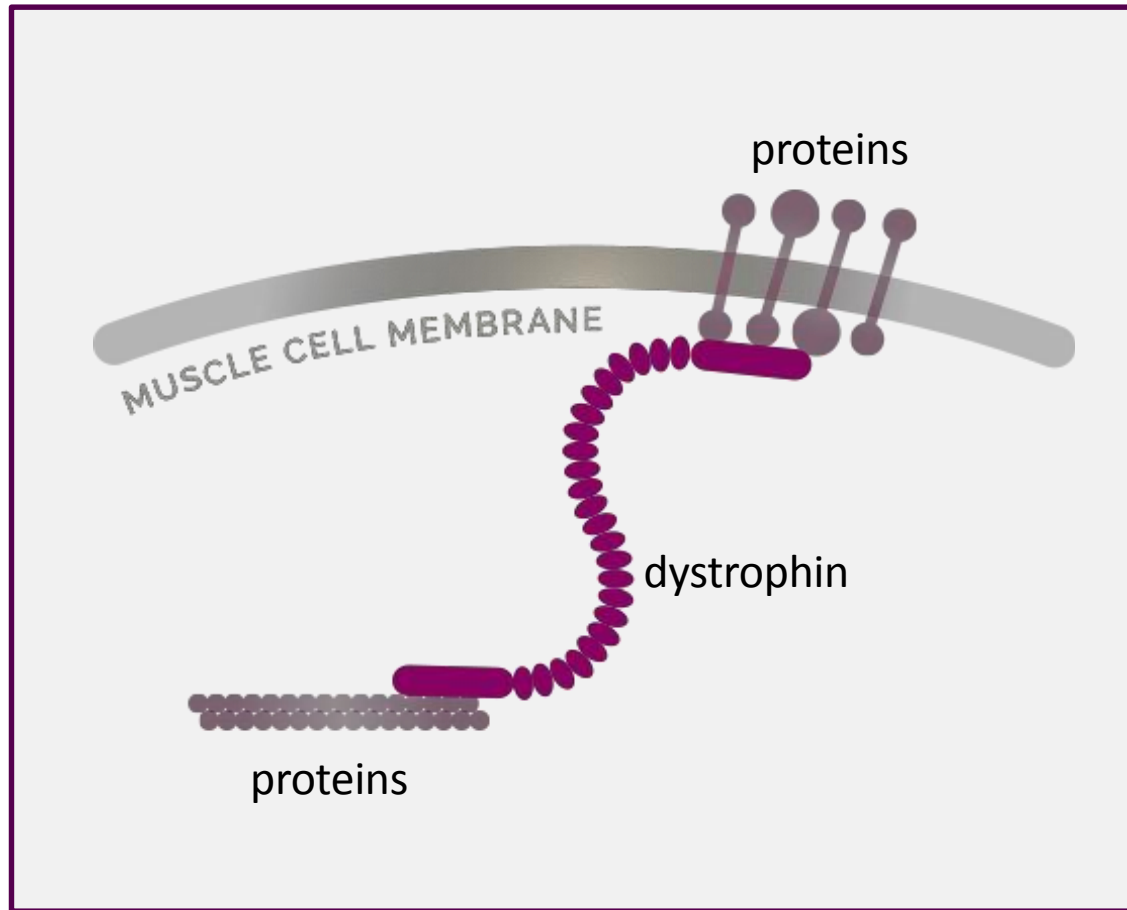
DISEASE PROGRESSION IN DMD³⁻⁶



3. Wong BL, Christopher C. Corticosteroids in Duchenne muscular dystrophy: a reappraisal. *J Child Neurol.* 2002;17(3):183-190
4. Bushby K, Finkel R, Birnkrant DJ, et al. *Lancet Neurol.* 2010;9:77-93.

5. Emery AEH. *Lancet.* 2002;359:687-695.
6. Landfeldt E, Lindgren P, Bell CF, et al. *Neurology.* 2014;83(6):529-536.

LACK OF DYSTROPHIN IS THE PROXIMATE CAUSE OF DMD



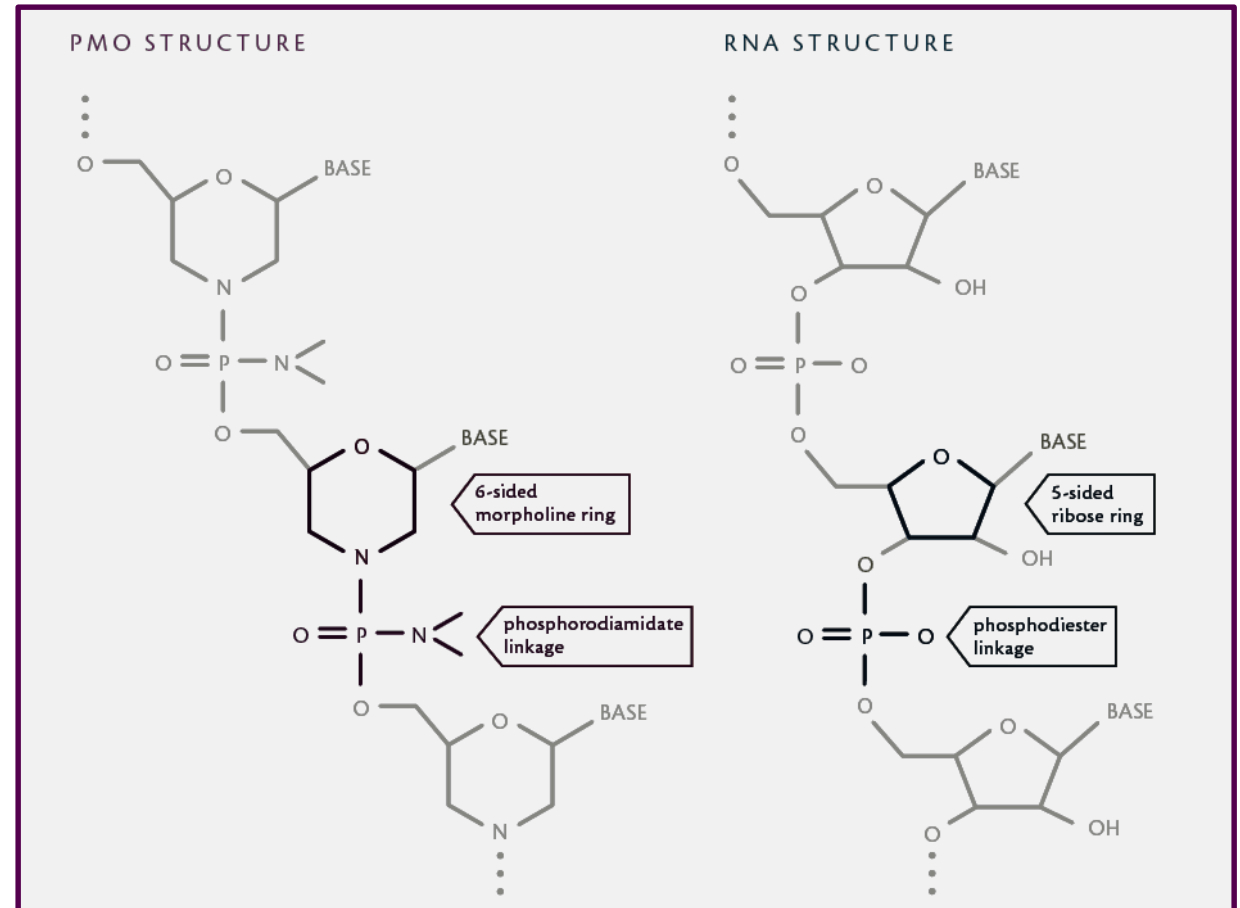
- Dystrophin is a protein found in muscle cells that helps strengthen and protect muscle fibers
- Because of an error in the genetic instructions, production of dystrophin protein is disrupted, which can result in DMD
- Without dystrophin, muscle cells are damaged, and, over time, are replaced with scar tissue and fat in a process called fibrosis

PHOSPHORODIAMIDATE MORPHOLINO OLIGOMER (PMO) TECHNOLOGY

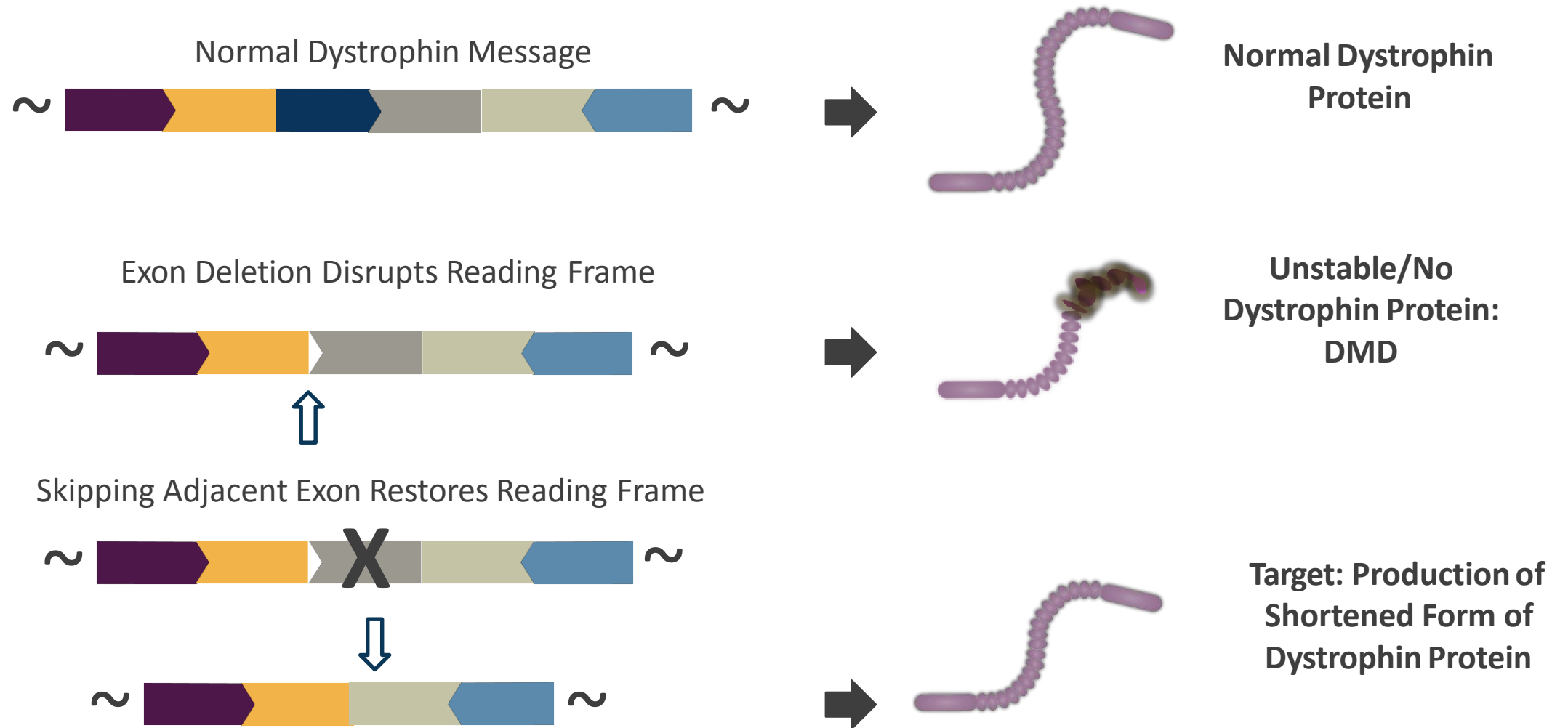
SYNTHETIC CHEMICAL STRUCTURES MODELED AFTER THE NATURAL FRAMEWORK OF RNA

Platform based on pioneering work with unique properties of the chemical structures

- Synthetic chemical structures, which are a re-design of natural RNA
- The morpholine rings are connected to each other by uncharged phosphorodiamidate linkages instead of the phosphodiester linkages found in RNA



EXON-SKIPPING RESTORES THE READING FRAME



COMMERCIAL OVERVIEW



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

POTENTIAL TO TREAT 13 PERCENT OF THE DMD PATIENT POPULATION

- ✓ Granted accelerated approval by U.S. Food and Drug Administration in September 2016
- ✓ Indicated for the treatment of DMD in patients amenable to exon 51 skipping
- ✓ Targets dystrophin deficiency, the underlying cause of DMD
- ✓ Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials



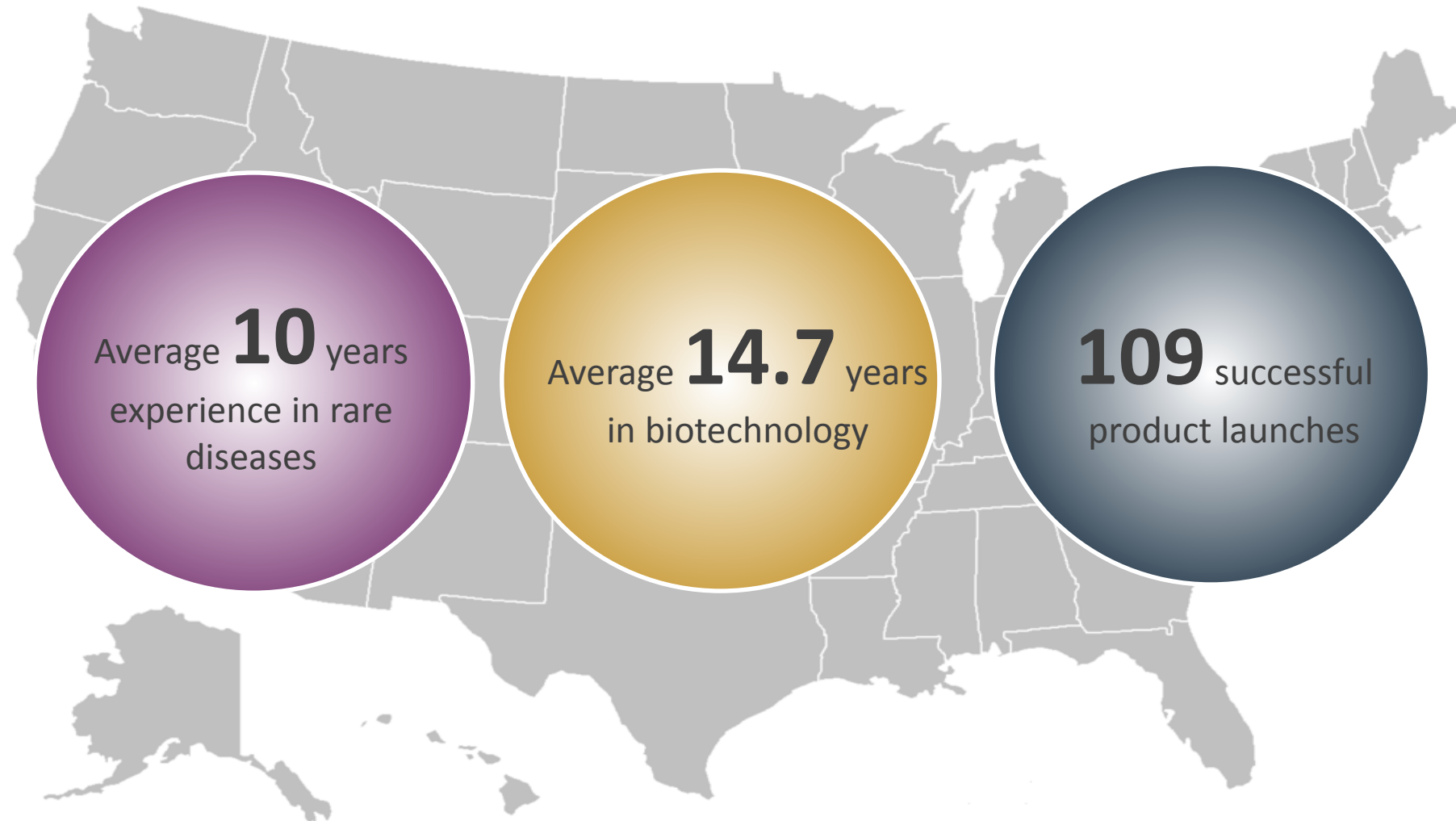
PATHWAY TO EXECUTING A SUCCESSFUL EXONDYS 51 LAUNCH

FOUR KEY ACTIVITIES



SAREPTA THERAPEUTICS' U.S. FIELD FORCE IN PLACE

EXPERIENCED REPRESENTATIVES ACROSS THE COUNTRY



FINANCIAL HIGHLIGHTS

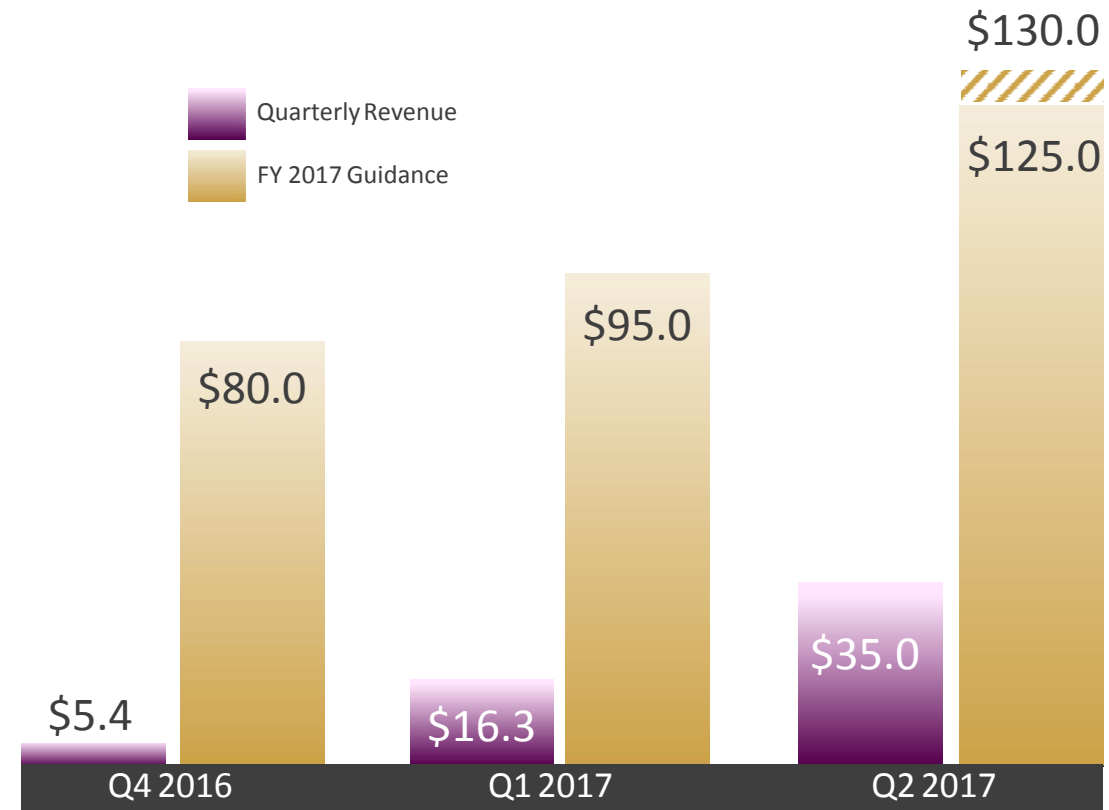
Successful launch of EXONDYS 51 with over 150 physicians submitting a START Form

Q2 2017 revenue of \$35 million versus \$16 million in Q1 2017

Raised full-year 2017 revenue guidance to \$125 to \$130 million from \$95 million

\$302 million in cash equivalents, restricted cash and investments at June 30, 2017

\$354 million in net proceeds from July 2017 public offering



GLOBAL EXPANSION



SECURED A GLOBAL PATENT ESTATE

BENEFITS FOR SAREPTA THERAPEUTICS

Worldwide freedom to operate for EXONDYS 51 and future exon-skipping products

Exclusive license to two robust exon-skipping patent estates positioning us well in the DMD landscape

Enhance and streamline the development of future exons

- Provides flexibility to utilize the most active sequences for drug candidates

Removal of uncertainty

Short duration: Payments for exon 51, 45, and 53 products end in 2023/24

MANAGED ACCESS PROGRAM (MAP)



Initiated July 2017
in Europe, North
and South America

Currently in 28
countries with
plans to expand

Expect to realize
revenue in late
Q4'17

EXONDYS 51: EUROPEAN SUBMISSION (MAA)

Proposed Indication

EXONDYS is indicated for treatment of Duchenne muscular dystrophy (DMD) in adults, adolescents and children aged 4 years and older who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping

Clinical Data

• Eteplirsen vs Untreated External Control

(exon 51 amenable patients)

- 4-Year 6MWT Data
- 4-Year Loss of Ambulation Data
- Supportive Data on other Outcome Measures
 - 3-Year North Star Ambulatory Assessment Total Score
 - 3-Year Ability to Rise Independently from Supine
 - Pulmonary Function (compared to literature)

• Eteplirsen vs. Secondary External Control

(n=50 any exon amenable patients)

- 3-Year 6MWT Data
- 3-Year Loss of Ambulation Data

Dystrophin (Week 180 vs. untreated controls)

- Percent Dystrophin Fibers
- Intensity
- Western Blot

Safety Database: N=150 (81 with ≥ 1 year of exposure)

PREPARING TO RAPIDLY COMMERCIALIZE IN EU, IF APPROVED

EMA REVIEW IS ANTICIPATED TO BE COMPLETED IN 1H 2018

EU commercial plans underway

Building out infrastructure and footprint

- Head of EU Operations hired
- EU headquarters in Zug, Switzerland opened
- Scaling manufacturing to meet potential demand
- Market research and KOL mapping completed
- IP/legal initiatives settled

Actively engaged with EU KOLs

Obtaining input from top EU DMD experts; planning strategic presence at major conferences

- World Muscle Society
- ICNMD: Symposium
- Action Duchenne
- EMA DMD Guidelines
- CNS

Ongoing clinical trials in the EU

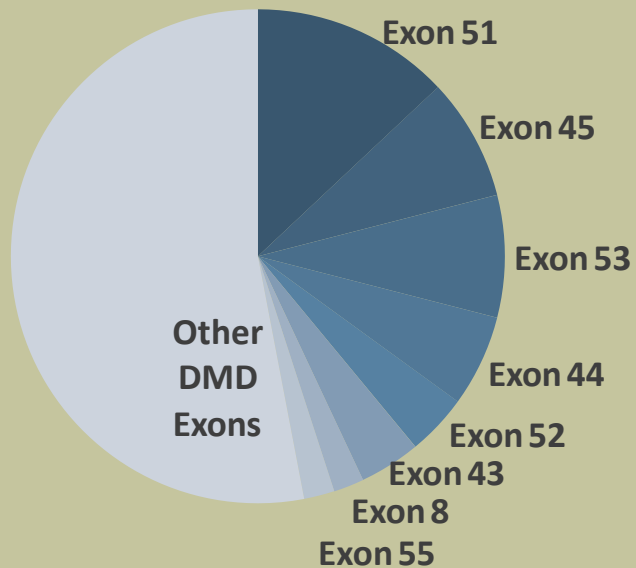
- SRPT 4053-101
- ESSENCE

CLINICAL DEVELOPMENT
PIPELINE:
EXON-SKIPPING PROGRAMS



DMD POPULATIONS AMENABLE TO EXON-SKIPPING THERAPIES

8 exons ≈ half
of all DMD patients



- 13% of DMD
- >3,400 patients
- 1 exon
 - Exon 51

- 29% of DMD
- >7,600 patients
- 3 exons
 - Exon 45
 - Exon 53
 - Exon 51

- 47% of DMD
- >12,000 patients
- 8 exons
 - Exon 44
 - Exon 52
 - Exon 43
 - Exon 8
 - Exon 55
 - Exon 45
 - Exon 53
 - Exon 51

ESSENCE STUDY: FOCUS ON 16 PERCENT OF THE DMD POPULATION WORLDWIDE

A GLOBAL PHASE 3 PLACEBO-CONTROLLED TRIAL FOR EXONS 45 AND 53 CURRENTLY ENROLLING

Applying Key Learnings from Eteplirsen Clinical Trials

Developed to enroll boys in whom a potential treatment effect might be most readily detected

- Age-range reduced from ages 7-16 to age 7-13
- Boys older than 13 years, who walk further than 300 meters on 6MWT have a less predictable disease course
- Allows for a more homogenous population

Lengthened from 1 to 2 years based on Sarepta's understanding of the time frame when a potential treatment effect on 6MWT might first be seen

Overview

- ~ 99 males to enroll (age 7-13)
- 96 week, double-blind, placebo controlled
- Roll over to open-label for 96 weeks
- Randomized 2:1 (66 active treatment:33 placebo)
- Conducted at sites in the U.S. and Europe



SEPTEMBER 2017: POSITIVE TOP-LINE RESULTS OF GOLODIRSEN (SRP-4053) IN PATIENTS AMENABLE TO SKIPPING EXON 53

Golodirsen uses Sarepta Therapeutics' proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 53 of the DMD gene

About the 4053-101 study

A Phase 1/2 first-in-human study conducted in Europe to assess the safety, tolerability, pharmacokinetics, and efficacy of golodirsen in 25 DMD patients with confirmed deletions of the DMD gene amenable to skipping exon 53

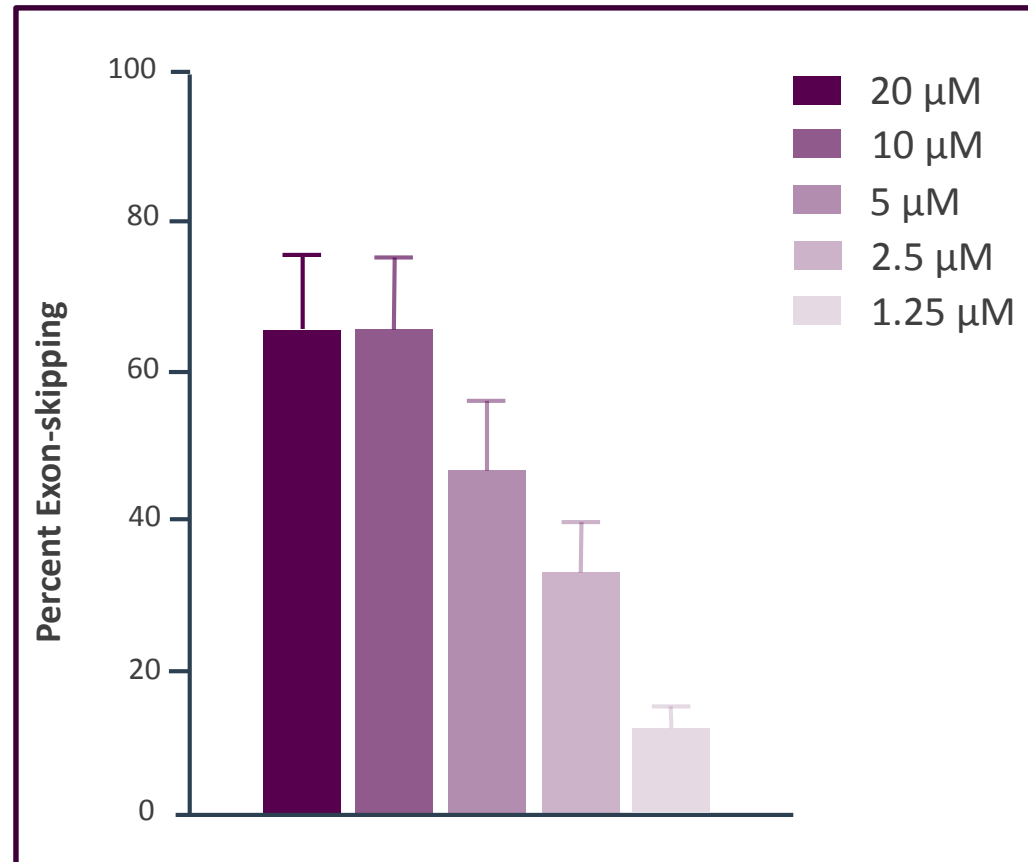
Study achieved statistical significance on all primary and secondary biological endpoints

Data demonstrated statistically significant exon skipping, dystrophin production and localization

Results further validate Sarepta Therapeutics' exon-skipping platform for the treatment of DMD

EXON 53 SKIPPING EFFICIENCY IN MYOBLASTS

Greater than 60 percent exon-skipping at the 10 μ M and 20 μ M



INTERNAL EXON-SKIPPING THERAPIES IN DEVELOPMENT

GENERATING A ROBUST BODY OF CLINICAL DATA

Study name	Trial design*	Status
ESSENCE Exon 45 or 53	96-week double-blind, placebo-controlled, multi-center study w/ an open-label extension to evaluate SRP-4045 and SRP-4053 versus placebo in DMD patients amenable to exon 45 or 53 skipping, respectively	Currently enrolling
PROMOVI Exon 51	96-week open-label, multi-center study to provide confirmatory evidence of efficacy of eteplirsen in DMD patients amenable to skipping exon 51	Completed enrollment
4658-203 Exon 51	96-week open-label, multi-center study to evaluate the safety, effectiveness, and tolerability of eteplirsen in early-stage DMD	Completed enrollment
4658-US-202 Exon 51	240+ week open-label, multiple-dose study to assess efficacy, safety, and tolerability of an additional 212 weeks of treatment with eteplirsen in patients who successfully completed study 4658-US-201	Completed enrollment
4658-204 Exon 51	96-weeks open-label, multi-center study w/ an open-label extension to evaluate the safety and tolerability of eteplirsen in patients with advanced-stage DMD	Completed enrollment
4053-101 Exon 53	Two-part, randomized, double-blind, placebo-controlled, dose-titration, safety, tolerability, and PK study (part 1) followed by an open-label efficacy and safety evaluation (part 2) of SRP-4053 in patients with DMD amenable to exon 53 skipping	Completed enrollment
4045-101 Exon 45	Randomized, double-blind, placebo-controlled, dose-titration, safety, tolerability, and PK study followed by an open-label safety and efficacy evaluation of SRP-4045 in advanced-stage DMD patients amenable to exon 45 skipping	Completed enrollment
4658-102 Exon 51	96-weeks, open-label safety, tolerability, and pharmacokinetics study of eteplirsen in young DMD patients (6-48 months) amenable to exon 51 skipping	Currently enrolling

NOTE: Does not include pending trial design under discussion with FDA exploring alternate dosing regimen(s)

*Original protocol design

CLINICAL DEVELOPMENT PIPELINE:
NEXT-GENERATION EXON-SKIPPING
PROGRAMS



PEPTIDE PHOSPHORODIAMIDATE MORPHOLINO OLIGOMER (PPMO): NEXT-GENERATION CHEMISTRY OVERVIEW

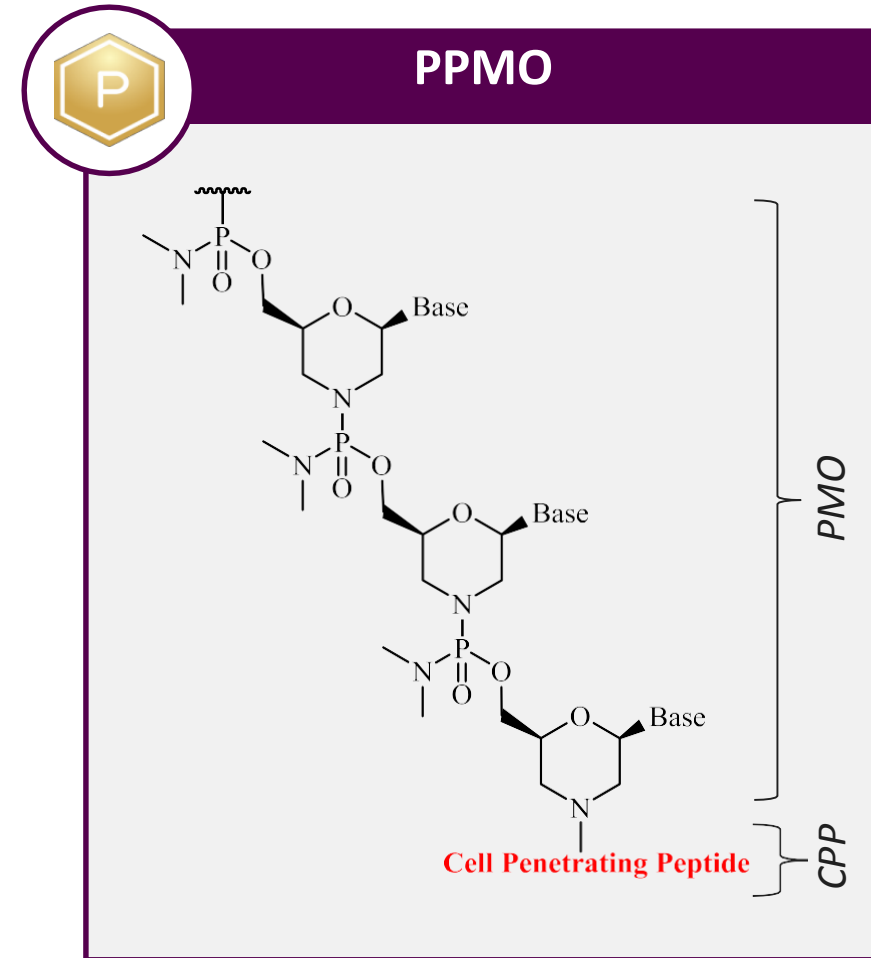
DESIGNED TO ENHANCE TISSUE TARGETING, INTRACELLULAR DELIVERY, SELECTIVITY AND POTENCY

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

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

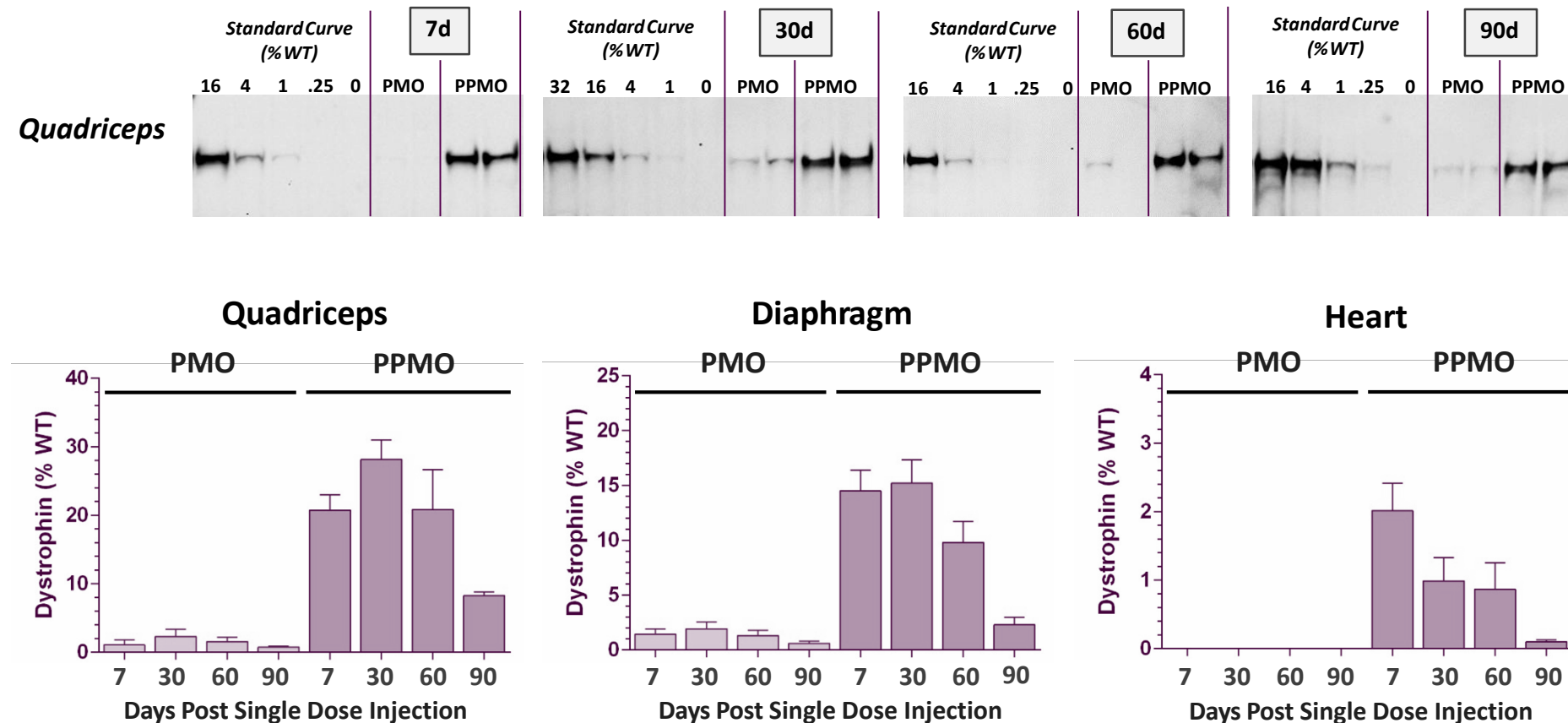
Proprietary class of PPMO compounds have the potential to provide:

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



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

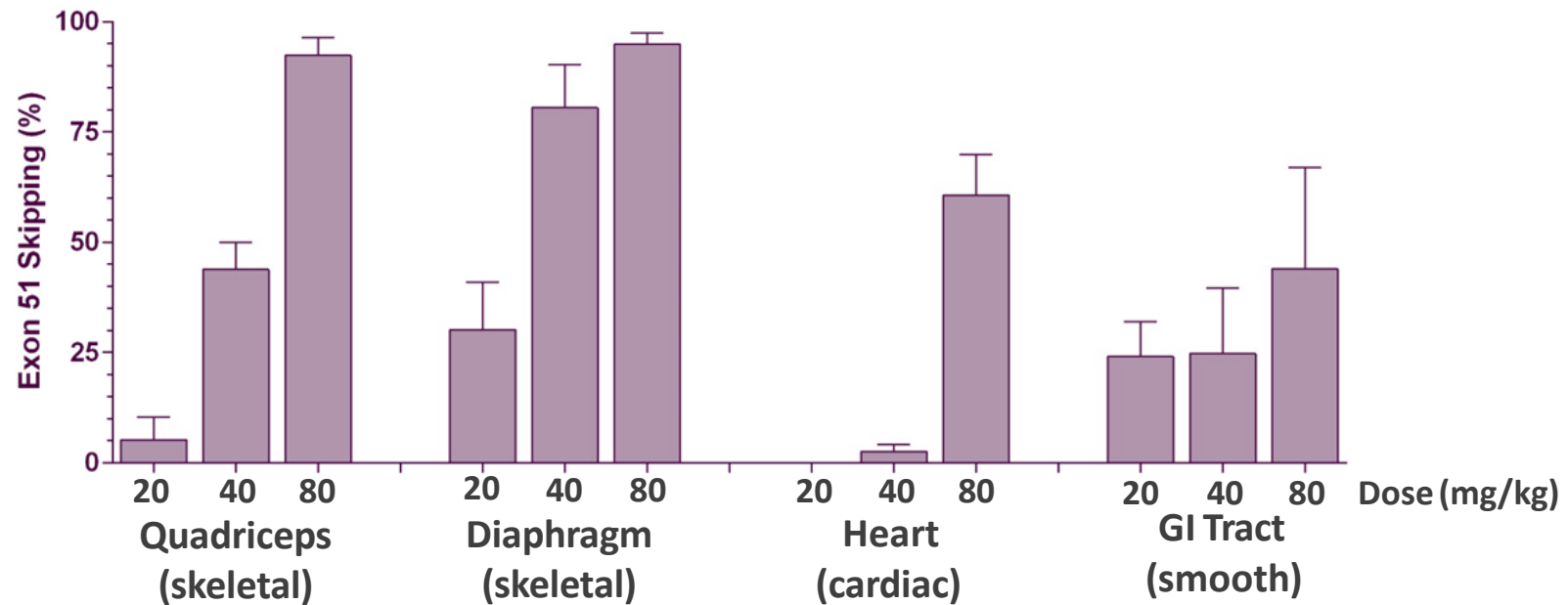
RESPONSE DURABILITY SUPPORTS LESS FREQUENT DOSING



- *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

PPMO CLINICAL CANDIDATE ACHIEVES GLOBAL DELIVERY IN NON-HUMAN PRIMATE (NHP)

>90% EXON 51 SKIPPING



- 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

ACCELERATING DEVELOPMENT OF PPMO PROGRAM TOWARD CLINICAL TRIALS

PPMO demonstrates enhanced activity vs. PMO

- Significantly higher dystrophin production
- Durability of response should support less frequent dosing

Initial toxicology in mouse and non-human primate indicate a favorable therapeutic window

IND-enabling GLP toxicology studies began earlier in 2017; target: filing IND and initiating a study by year-end 2017

CLINICAL DEVELOPMENT PIPELINE: GENE THERAPY PROGRAMS



NATIONWIDE CHILDREN'S HOSPITAL (NCH): MICRO-DYSTROPHIN GENE THERAPY

In January 2017, Sarepta entered into a research and option agreement with NCH in Ohio for its micro-dystrophin gene therapy program

- Jerry Mendell, M.D. and Louise Rodino-Klapac, Ph.D. are the lead investigators
- Phase 1/2a trial to be initiated in fall of 2017 at NCH
- Parent Project Muscular Dystrophy (PPMD) committed \$2.2 million to the trial, which has support from additional DMD foundations and families

Micro-dystrophin gene therapy approach can target the majority of patients with DMD



NATIONWIDE CHILDREN'S HOSPITAL (NCH): GALGT2

In January 2017, Sarepta announced a license agreement with NCH for GALGT2 gene therapy program

- Aim is to activate genes that make proteins that can perform a similar function as dystrophin
- The goal is to produce a muscle cell that can function normally even when dystrophin is absent
- The program was developed by researcher Paul Martin, Ph.D. and is expected to enter the clinic as a Phase 1/2a trial in fall of 2017

Potential to treat all boys with DMD regardless of any underlying dystrophin mutation and other dystrophinopathies



GENETHON: MICRO-DYSTROPHIN GENE THERAPY

In June 2017, Sarepta and Genethon entered into a gene therapy research collaboration to jointly develop treatments for DMD

- Micro-dystrophin is a shortened version of dystrophin that can perform a similar function
- Genethon demonstrated robust gene expression in a GRMD model of DMD
- Data from this program was published in the July 25, 2017 online issue of *Nature Communications*
- Genethon is responsible for early development; Sarepta has option for exclusive U.S. rights

Micro-dystrophin gene therapy approach can target the majority of patients with DMD



CLINICAL DEVELOPMENT PIPELINE:
UTROPHIN MODULATION PROGRAM



SUMMIT THERAPEUTICS: UTROPHIN MODULATION

In October 2016, Sarepta entered into an exclusive license and collaboration agreement for European rights to Summit's utrophin modulator pipeline for the treatment of DMD

- Summit received \$40 million upfront, with potential future milestone payments plus royalties
- Sarepta and Summit to share R&D costs at a 45 / 55 percent split beginning in 2018
- The program is currently in a Phase 2 proof-of-concept trial (24-week data readout expected in Q1'18; top-line data from the complete 48-week trial expected in Q3'18)

Utrophin modulation is a potential disease-modifying treatment for all DMD patients



NEAR-TERM MILESTONES



SEVERAL NEAR-TERM, VALUE-CREATING MILESTONES

*Global leader in the discovery,
development and commercialization
of DMD therapies*

COMMERCIAL

- Early U.S. launch success
- Expansion into EU and other regions
- Managed Access Program launched

REGULATORY

- Marketing Authorization Application
- U.S. confirmatory studies
- Filing INDs

CLINICAL

- Up to seven programs in the clinic by YE'17
- Late-stage development
- Rapidly advancing pipeline



THANK YOU



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