



SAREPTA

THERAPEUTICS

RNA-TARGETED PRECISION
MEDICINE FOR DUCHENNE
MUSCULAR DYSTROPHY

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: The market opportunity for DMD in and Sarepta's goals to expand into Europe and other regions; the potential role and impact of small amounts of dystrophin on DMD disease progression; Sarepta's beliefs that it has established a strong foundation for the Exondys 51 launch and its plans to continue building on it, that the early stage of the launch and access discussions with payors are going well and that they will ultimately cover the majority of patients who have submitted a start form, that the initial quarter of this launch is highly competitive with those of some of the most successful launches for ultra-rare diseases, that Sarepta is well positioned for success in 2017 and its plans for building shareholder value in 2017 by among other things executing on Sarepta's identified key areas of focus to support the launch, its strategic plans with payors, and Sarepta's expectation that conversion rates will gradually accelerate throughout the second quarter and the rest of the year; Sarepta's plans for its pipeline and technology, including PPMO, development efforts, partnerships and their respective potential benefits; Sarepta's Q4 2016 net revenue (unaudited) and other financial expectations; Sarepta's strategic and commercialization plans in the EU for Exondys 51 and Sarepta's belief that it has submitted data supporting the clinical efficacy of Exondys 51 and expectations relating to the EMA's Exondys 51 MAA evaluation process and timelines; Sarepta's belief that its robust clinical development program, including ESSENCE, could form a potential foundation for an accelerated clinical development pathway for follow-on therapies; and other statements made during the presentation regarding Sarepta's future, strategy and business plans.

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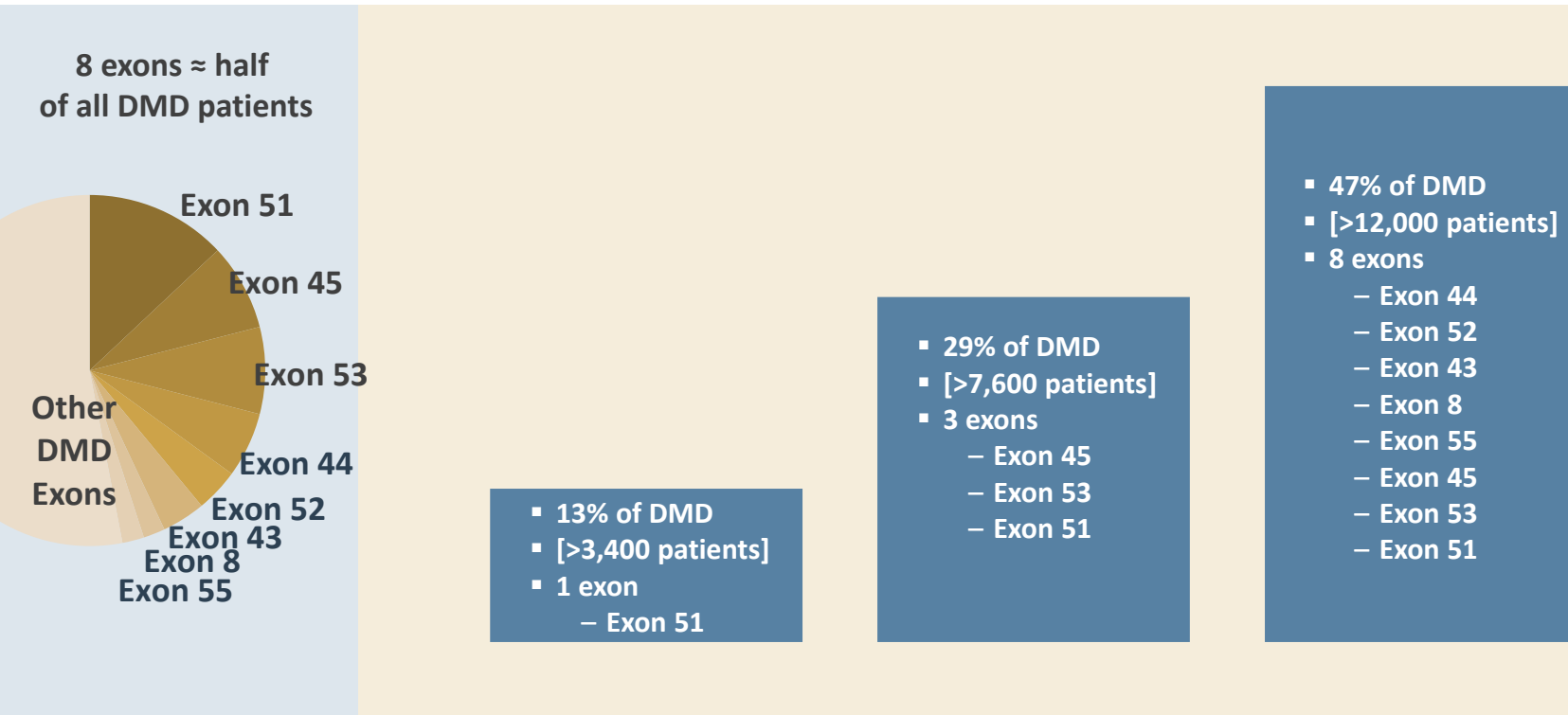
WHAT IS DUCHENNE MUSCULAR DYSTROPHY?

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



- Rare progressive neuromuscular genetic disease; fatal with average lifespan of mid to late 20s
- Diagnosis occurs between ages 4-5
- Caused by gene mutation that encodes for dystrophin – a protein that plays a key structural role in muscle fiber production
- Small amounts of dystrophin may change the progression of the disease

DMD REPRESENTS A SIGNIFICANT OPPORTUNITY IN KEY MARKETS AROUND THE WORLD



BUILDING VALUE FOR SHAREHOLDERS IN 2017

3. Accelerate Multiple Treatment Approaches

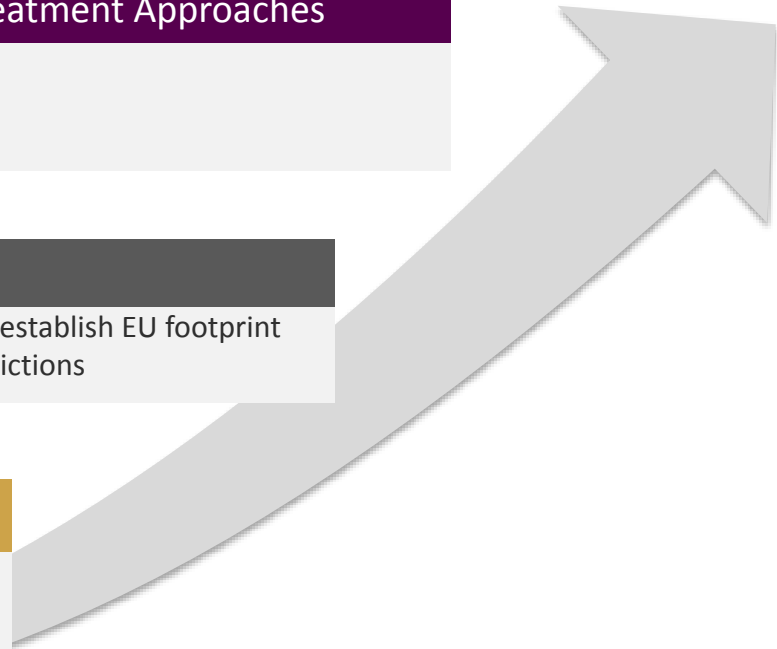
- Exon 45&53
- PPMO
- 4 External Collaborations

2. Global Expansion

- Pursue and secure EMA approval and establish EU footprint
- Prepare for early access in other jurisdictions

1. Achieve Successful Commercial Launch

- ✓ EXONDYS 51 launch in US
- ✓ Increase awareness and importance of knowing your mutation

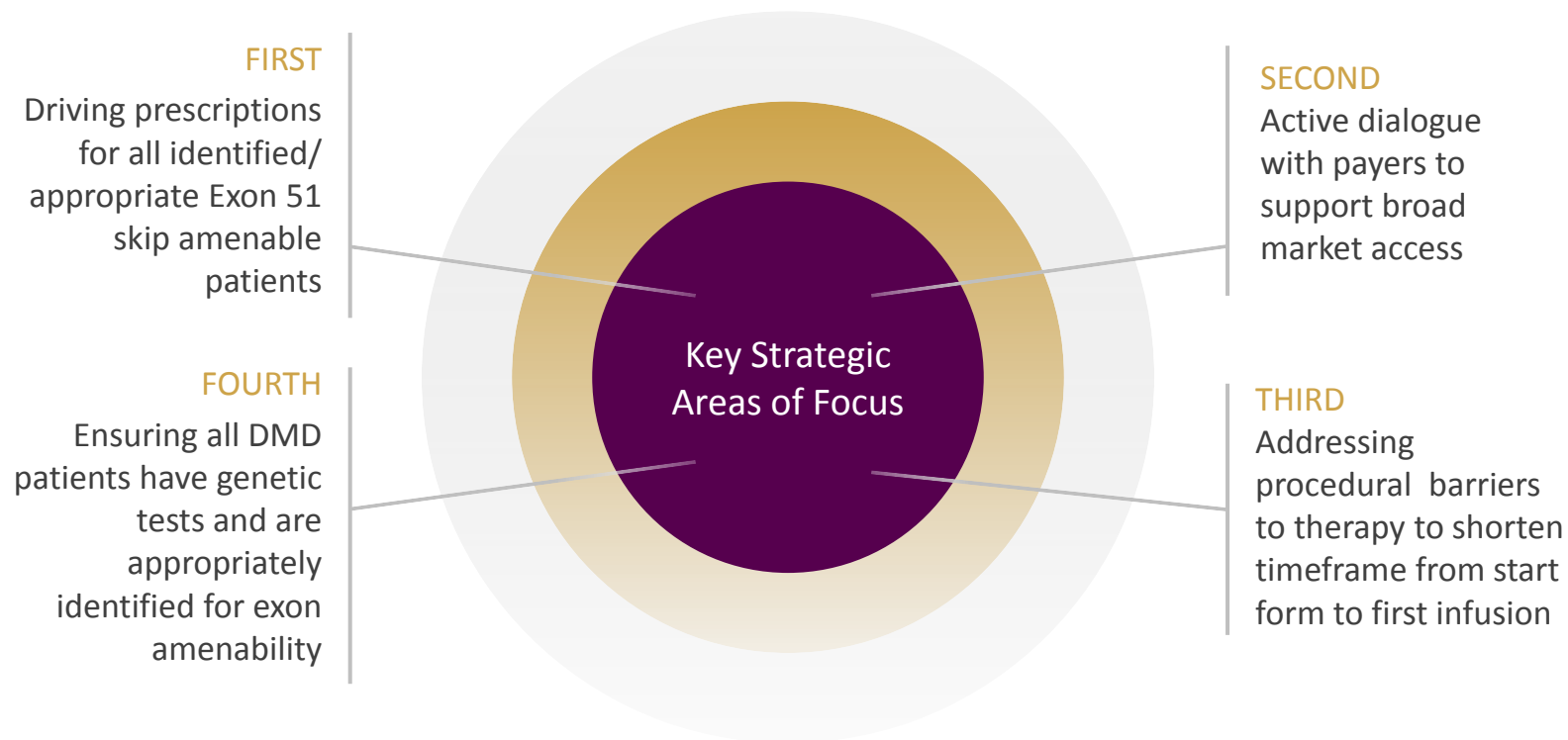


EXONDYS 51™
COMMERCIAL UPDATE



DRIVING A SUCCESSFUL EXONDYS 51 LAUNCH

FOUR KEY ACTIVITIES



STRONG INITIAL PHYSICIAN AND PATIENT DEMAND

COMMERCIAL EXECUTION: U.S. MARKET LAUNCH

**Genetically Verified Start Forms:
Over 250 Since Launch**

**Q4 Net Revenue (unaudited):
~\$5.4 Million**

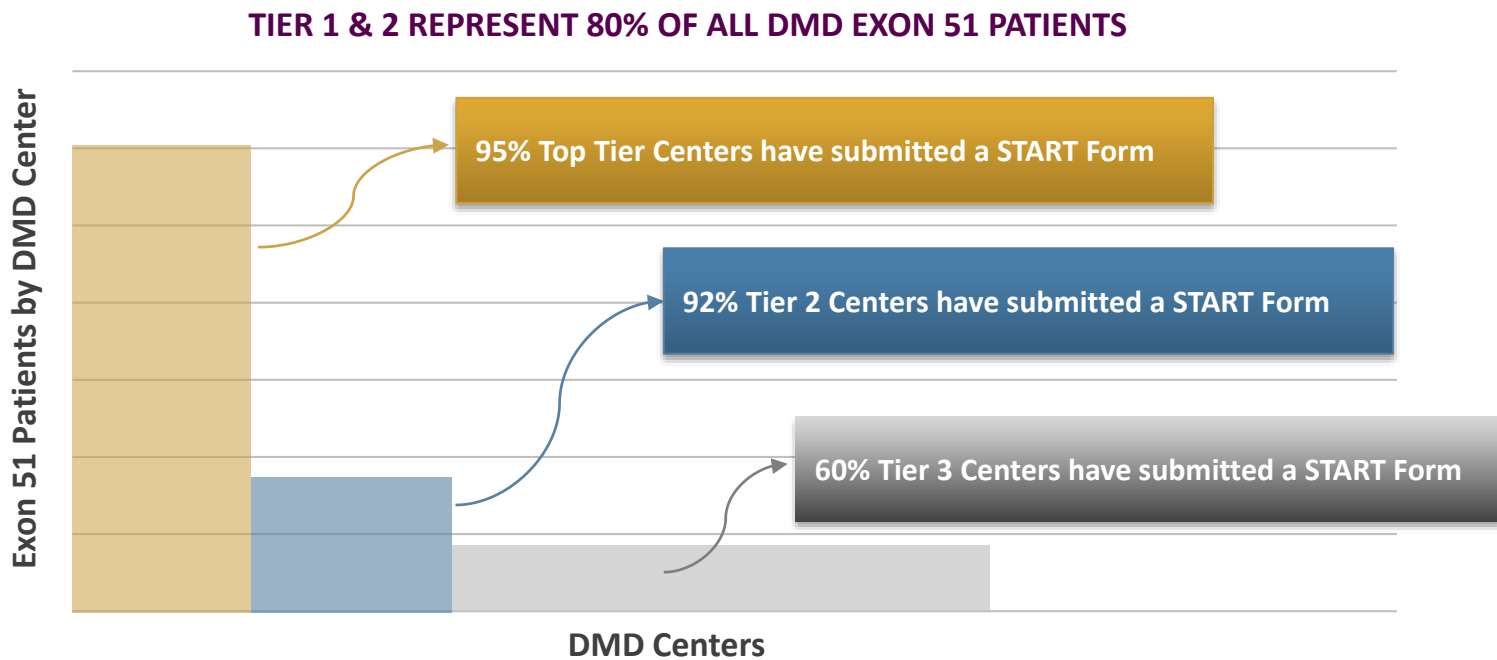
Patient Demographics

**Current Start Forms:
~60% commercial /
40% Medicaid**

**Age of Patients on
Therapy:
9 months - 27 years**

**Average Age of Patients
on Therapy:
13 years**

MAJOR DMD CENTERS ARE ACTIVELY SUBMITTING START FORMS



ACCESS DISCUSSIONS PROGRESSING WELL

**9 National Health Plans Covering
>156 Million Lives**

**22 Regional Accounts Covering
60 Million Lives**

**5 of the Top Commercial PBMs
3 of the Top Medicaid PBMs**

**13 of the Top Medicaid States
Covering 27 Million Lives**

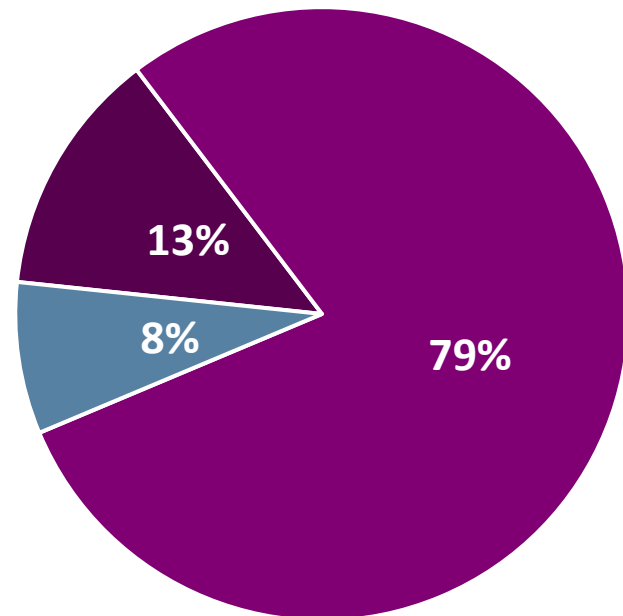
**Payers actively engaged in learning more about EXONDYS 51 (i.e. >25 AMCP
Dossier requests received to date, ~20 Medical / MSL presentations)**

MAJORITY OF PLANS COVERING OR EVALUATING COVERAGE CASE-BY-CASE

13% of Covered Lives have a favorable policy
and/or covering to USPI

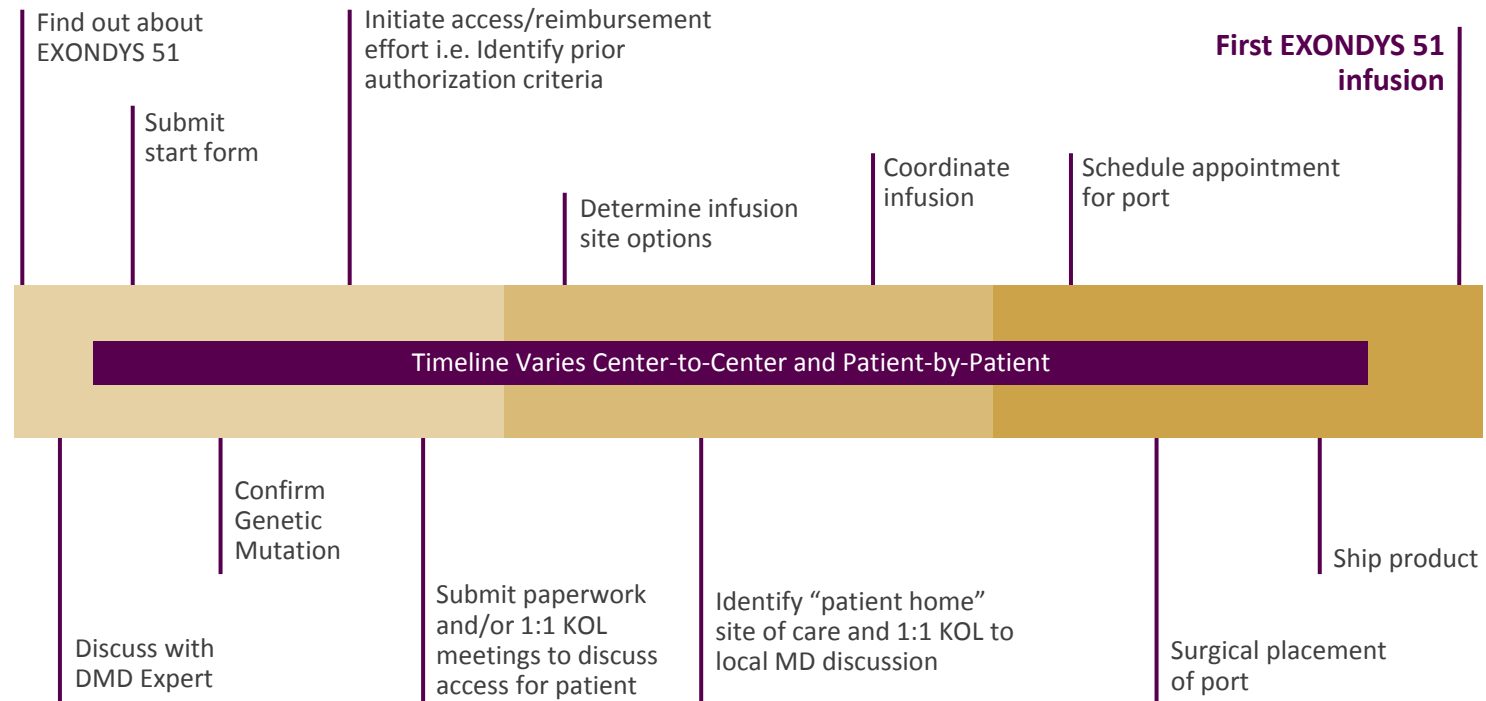
79% of Covered Lives are pending policy
decisions, reviewing case-by-case or
approved with restrictions

8% of Covered Lives are denying coverage
(conversations continue)



As of 12/31/16

PATIENT JOURNEY TO FIRST INFUSION



DRIVING PATIENT IDENTIFICATION THROUGH INCREASED GENETIC TESTING AND INTERPRETATION

INTERPRETATION

- PPMD partnership (Decode Duchenne genetic counselors)
- MDA supported DMD Chart Review Program
- Duchenne.com (Exon Deletion Tool)
- Genetic Mutation Worksheet

GENETIC TESTING

- PPMD partnership (Decode Duchenne support for testing)
- HCP “Importance of Genetic Testing” Brochure (Field driven)
- Duchenne.com patient education “knowing your DMD mutation”

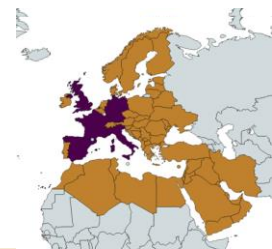
PPMD – DeCode Duchenne

- 7-fold increase year-over-year in testing applications
- Highest month of program to-date (last month)

TREATING MORE
BOYS WITH DMD



EUROPE IS A LARGE OPPORTUNITY



Larger exon 51 opportunity (patient numbers) than the U.S.

E.U. market research and KOL mapping completed

Strategic approach to E.U. congress management

IP legal initiatives progressing



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

1. 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

2. Dystrophin (Week 180 vs. untreated controls)

- Percent Dystrophin Fibers
- Intensity
- Western Blot

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

PREPARING TO RAPIDLY COMMERCIALIZE IN EU IF APPROVED

EU Commercial Plans Underway

- Building out infrastructure footprint
- Scaling manufacturing to meet potential demand for a European launch

Actively Engaged with EU KOLs

Ongoing advisory from top EU DMD experts and significant presence at major conferences

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

Active Clinical Trial Programs in EU

- SRPT 4053-101
- ESSENCE

ESSENCE WILL TARGET AN ADDITIONAL 16%

A GLOBAL PHASE THREE PLACEBO CONTROLLED TRIAL FOR EXONS 45 &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 our 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 US, Europe, Canada



ROBUST CLINICAL DEVELOPMENT PROGRAM

STUDY NAME & NO. EXON TARGET	LENGTH OF STUDY	NO. OF PATIENT	ENROLLMENT STATUS
4045-301 (ESSENCE) Exon 45 or Exon 53	96 weeks (plus 96 week extension)	~99	Enrolling
4658-301 (PROMOVI) Exon 51	48 weeks (plus 48 week extension)	~160 ~80 treated ~ 80 untreated	Enrolling (Treated & Untreated)
4658-203 Exon 51	96 weeks	~40 ~20 treated ~20 untreated	Enrolling (Untreated)
4658-US-202 Exon 51	240+ weeks	12	Complete
4658-204 Exon 51	96 weeks	~24 ~24 treated	Complete
4053-101 Exon 53	Part 1: 12 weeks Part II: 48 weeks	12 (Part I); 48 (Part II) Part I: 8 placebo, 4 treated Part II: 24 treated (12 Part I), 24 untreated	Complete
4045-101 Exon 45	12 weeks (plus 108 weeks extension)	~12 ~12 treated (extension phase)	Complete

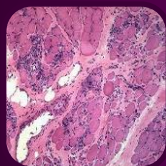
Does not include pending trial design under discussion with FDA exploring alternate dosing regimen(s)

STAGING A MULTI-FRONT
BATTLE AGAINST DUCHENNE



EXPANDING OUR PORTFOLIO OF DMD TREATMENTS

EXECUTED FOUR EXTERNAL PARTNERSHIPS



Muscle Fibrosis (Catabasis)

Exploring benefits of co-administration of exon skipping and NF-KB inhibition



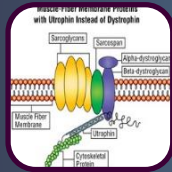
Exon Skipping

disease modifying backbone to address to underlying cause of DMD

Staging a multi-front battle against Duchenne

EXPANDING OUR PORTFOLIO OF DMD TREATMENTS

EXECUTED FOUR EXTERNAL PARTNERSHIPS



Utrophin Modulation (Summit)

Potential to be disease-modifying therapy for all underlying mutations of DMD



Exon Skipping

disease modifying backbone to address to underlying cause of DMD

Staging a multi-front battle against Duchenne

EXPANDING OUR PORTFOLIO OF DMD TREATMENTS

EXECUTED FOUR EXTERNAL PARTNERSHIPS



Gene Therapy (Nationwide Children's Hospital)

Partnering to advance **MicroDystrophin** gene therapy into clinical trials



Exon Skipping

disease modifying backbone to address to underlying cause of DMD

Staging a multi-front battle against Duchenne

EXPANDING OUR PORTFOLIO OF DMD TREATMENTS

EXECUTED FOUR EXTERNAL PARTNERSHIPS



Gene Therapy (Nationwide Children's Hospital)

Partnering to advance GalgT2 gene therapy into clinical trials



Exon Skipping

disease modifying backbone to address to underlying cause of DMD

Staging a multi-front battle against Duchenne

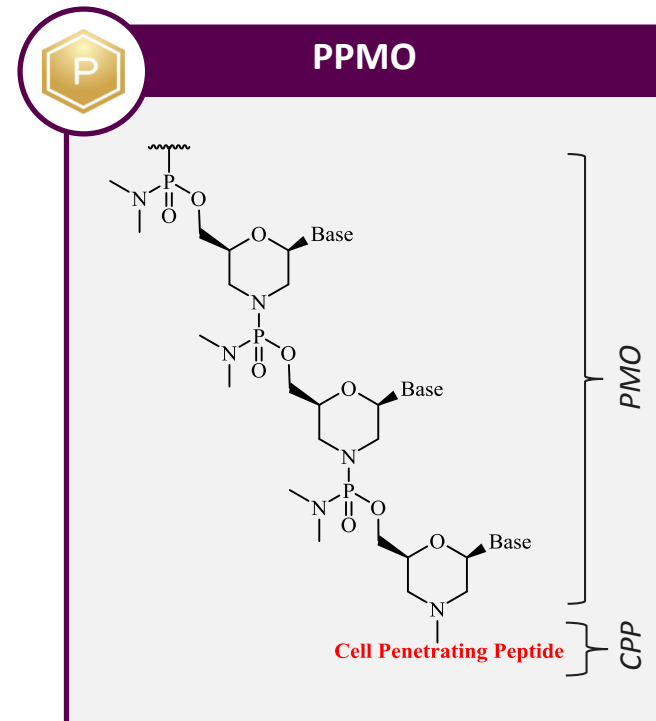
NEXT GENERATION PRE-
CLINICAL DATA



PPMO: NEXT GENERATION ANTISENSE PLATFORM TO TARGET MUSCLE AND OTHER ORGANS

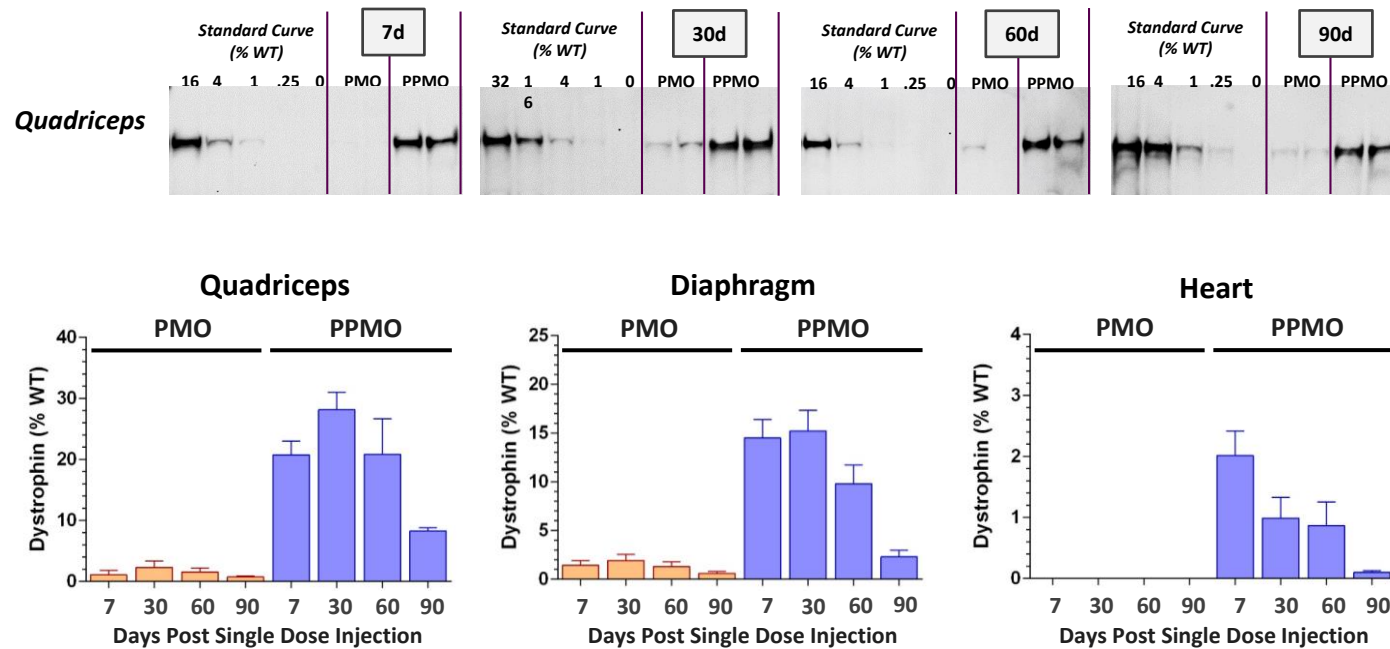
Proprietary class of PPMO compounds have the potential to provide:

- Improved delivery *in vivo*
- Superior dystrophin production *in vivo*
- Tolerability in non-human primates
- Less frequent dosing
- Extended patent term
- A platform technology that can potentially be tailored to target any organ



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

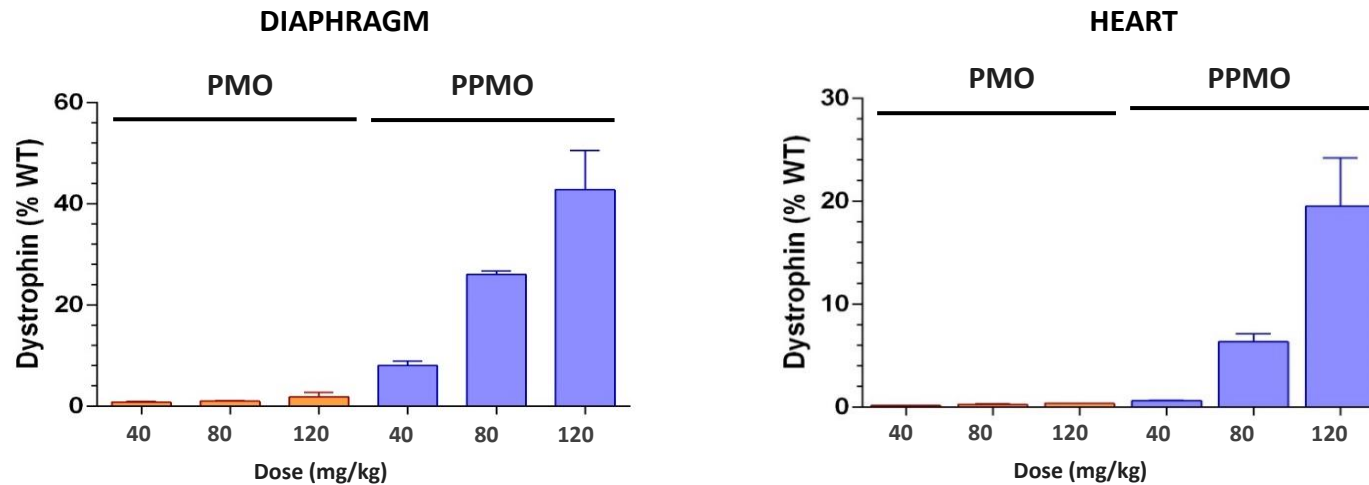
RESPONSE DURABILITY SUPPORTS LESS FREQUENT DOSING



- *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

A SINGLE PPMO DOSE INCREASES DYSTROPHIN LEVELS IN A DOSE-DEPENDENT MANNER IN MDX MICE

CELL PENETRATING PEPTIDE INCREASES POTENCY VS. PMO WITH MATCHED SEQUENCE



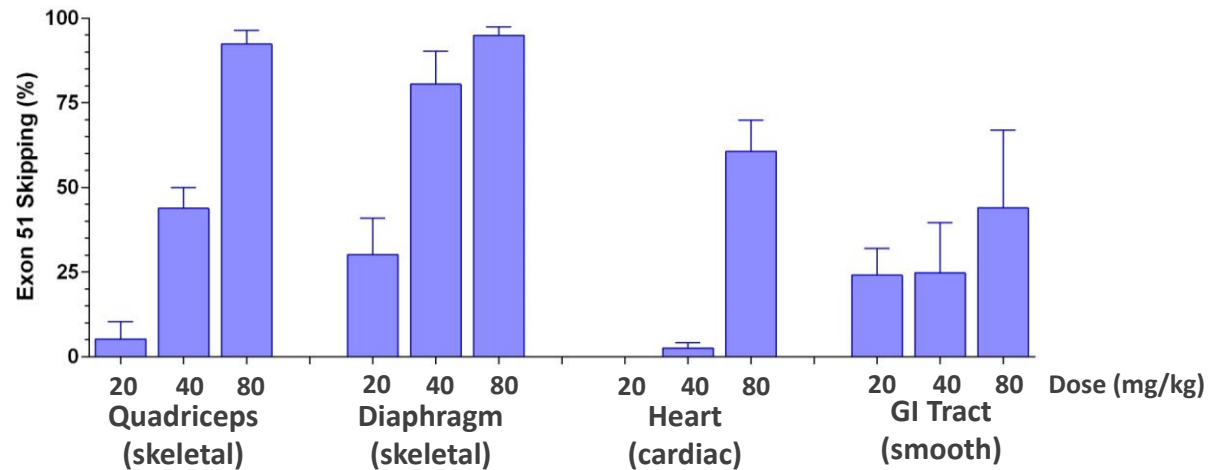
Dose Response:

Levels of dystrophin at 30 days post single dose injection of 40, 80, or 120 mg/kg

- *mdx* (DMD) mice were treated with a single IV dose of PMO or PPMO (with the clinical cell penetrating peptide)
- The **clinical Western blot method for dystrophin** was performed on muscle at 30 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 TOWARDS CLINICAL TRIALS

PPMO demonstrates enhanced efficacy vs. PMO

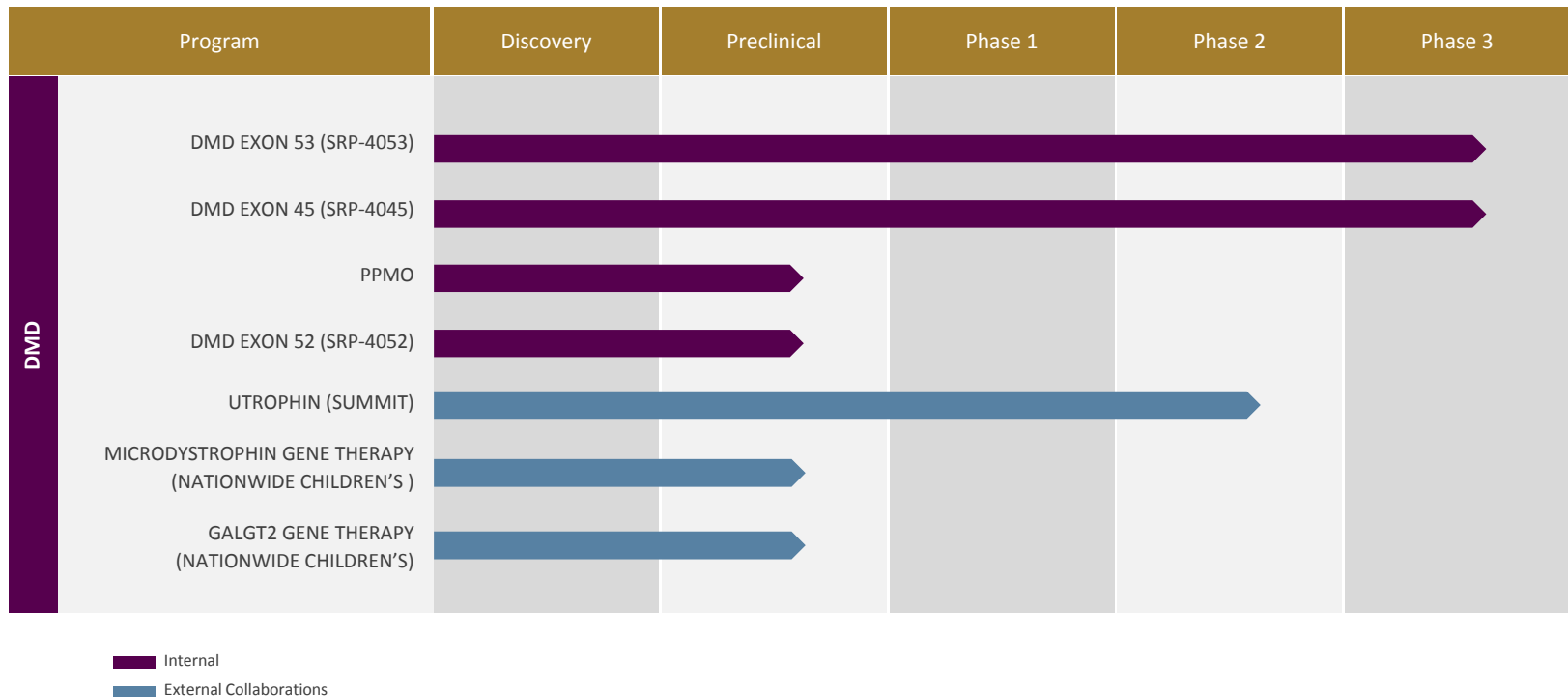
- Significantly higher dystrophin production
- Durability of response should support less frequent dosing

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

IND-enabling GLP toxicology studies to begin early this year; target opening IND before year-end 2017

COMPREHENSIVE APPROACH TO TREATING DMD

UP TO 7 PROGRAMS IN CLINIC IN 2017



SAREPTA WELL POSITIONED FOR SUCCESS IN 2017

Sarepta will be the global leader
in RNA-targeted precision
medicine to treat DMD



**EARLY US LAUNCH
SUCCESS**

**EXPANSION INTO EUROPE
AND OTHER REGIONS**

Rapidly advancing pipeline:

- Exons 45/53
- PPMO
- 4 External collaborations

THANK YOU



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