SAREPTA
THERAPEUTICS

Precision genetic medicine

for rare neuromuscular diseases

36TH ANNUAL JP MORGAN HEALTHCARE CONFERENCE
SAN FRANCISCO, CALIFORNIA

JANUARY 8, 2018
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’s aspiration to be a global top-five rare-disease focused company, applying its expertise in precision genetic medicine to address a variety of neuromuscular conditions; Sarepta having all the elements in place for success; Sarepta’s goal to develop life-changing precision genetic medicines to treat 100% of individuals with DMD and apply its therapeutic approach to other rare neuromuscular diseases; Sarepta’s new clinical studies evaluating potential life-changing modalities; Sarepta’s raised capital intending to accelerate R&D, expand talent base and support partnering; 2018 being a year of transformation for Sarepta; Sarepta’s key milestones and inflection points for 2018, including FDA meeting on golodirsen in Q1, CHMP decision for eteplirsen in mid-2018, submitting an IND for SRP-5053 in H2 2018, and having certain readouts and generating data from studies; Sarepta’s plan to develop treatments for all eligible individuals with DMD; Sarepta building a global franchise in neuromuscular diseases and its’s product development strategy and possibilities; Sarepta’s pipeline, technologies and next-generation approaches and their respective potential benefits, including PPMO potentially being a transformative approach to treating DMD and slowing disease progression in DMD with potentially less frequent dosing in the clinic and application in other therapeutic areas, and the potential of Sarepta’s programs with its partners to address all individuals with DMD, including the promise of micro-dystrophin gene therapy in DMD, GALGT2 gene therapy’s potential to restore muscle function despite the absence of the dystrophin protein, and gene therapy’s potential to address 100% of eligible individuals with DMD; PMO’s potential to treat 29% of individuals with DMD by 2019, including EXONDYS 51’s target to treat 13% of the DMD population and golodirsen and casimersen’s potential to treat an additional 16% of DMD population; Sarepta’s potential for up to 7 approved therapies in the U.S. by 2022; Sarepta being well-positioned for future success; Sarepta’s base case of more than $2 billion potential peak sales for late-stage PMO therapies and such base case representing a significant opportunity, and the opportunities lying in Sarepta’s other PMO exons, PPMO, gene therapy and other potential therapeutic areas; Sarepta’s key drivers of success; Sarepta’s revenue from EXONDYS 51 in the fourth quarter of 2017 and in the year 2017, its anticipation for strong growth in 2018, its revenue guidance for 2018, and its anticipation of approximately 100% year-over-year revenue growth; 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: the audit of our financial statements for the year ended December 31, 2017 is ongoing and could result in changes to the information; we may not be able to meet expectations with respect to EXONDYS 51 sales or attain the net revenue we anticipate for 2018, profitability or positive cash-flow from operations; we may not be able to achieve our expected base case and the expected year-over-year revenue growth; 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; the results of our ongoing research and development efforts, including those with strategic partners, and clinical trials for our product candidates, including, PPMO, golodirsen, casimersen and gene therapy, 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 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, results of research and development efforts and/or clinical trials may not be positive, 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 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.

*Sarepta Therapeutics aspires to be a **global top-five rare-disease focused** company, applying its expertise in **precision genetic medicine** to address a variety of **neuromuscular conditions**.*

ALL THE ELEMENTS IN PLACE FOR SUCCESS

Multi-platform pipeline

Funded for the future

EXONDYS 51™
(eteplirsen) Injection

Strong foundation

Urgent mission

DRIVEN BY AN URGENT MISSION

Our goal is to develop life-changing precision genetic medicines to treat 100 percent of individuals with Duchenne muscular dystrophy (DMD) and apply our therapeutic approach to other rare neuromuscular diseases where it is likely to have the most benefit

2017: SETTING THE STAGE FOR SUCCESS

LAUNCH

EXECUTED

TOP 5

most successful U.S. rare disease launches based on first-year revenue

PARTNERING

SIGNED

4

collaborations for next-generation precision genetic therapies

R&D

INITIATED

3

clinical studies evaluating potential life-changing modalities

FINANCIAL

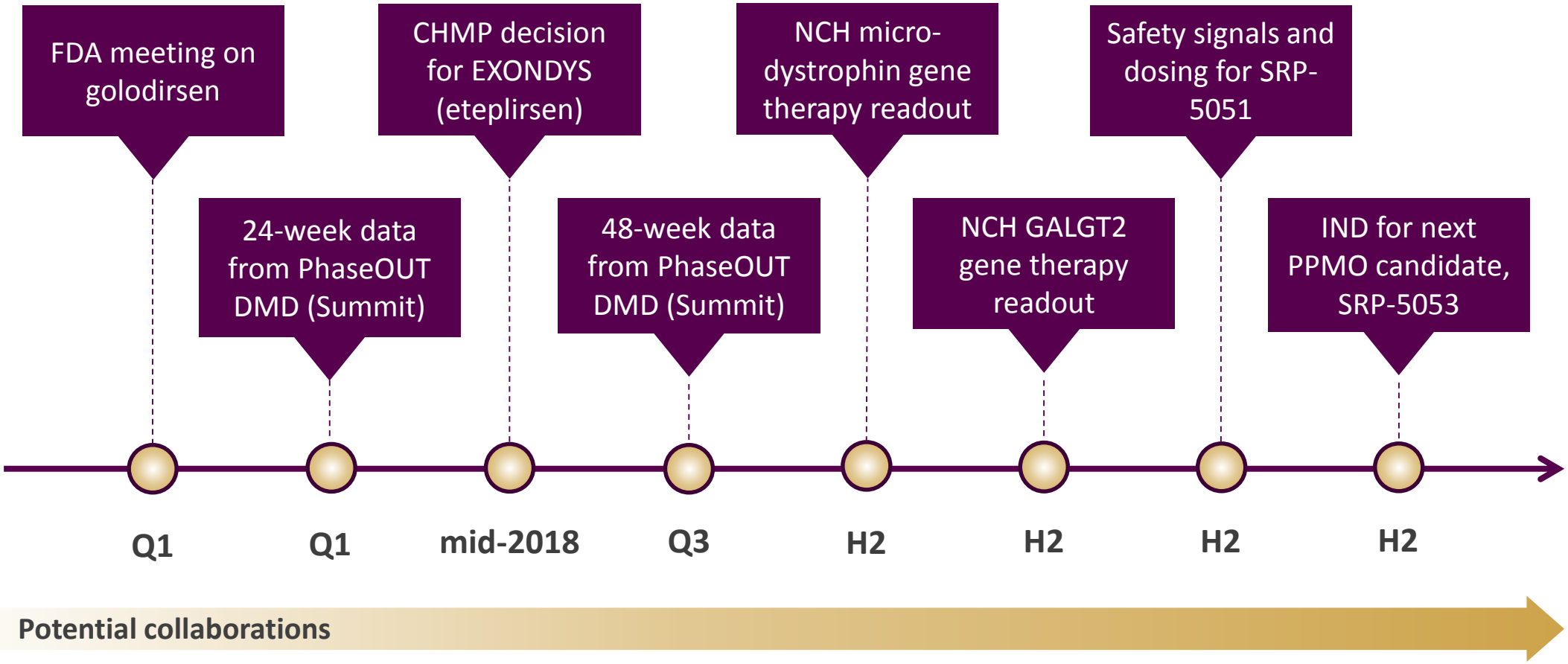
ADDED

>$1B

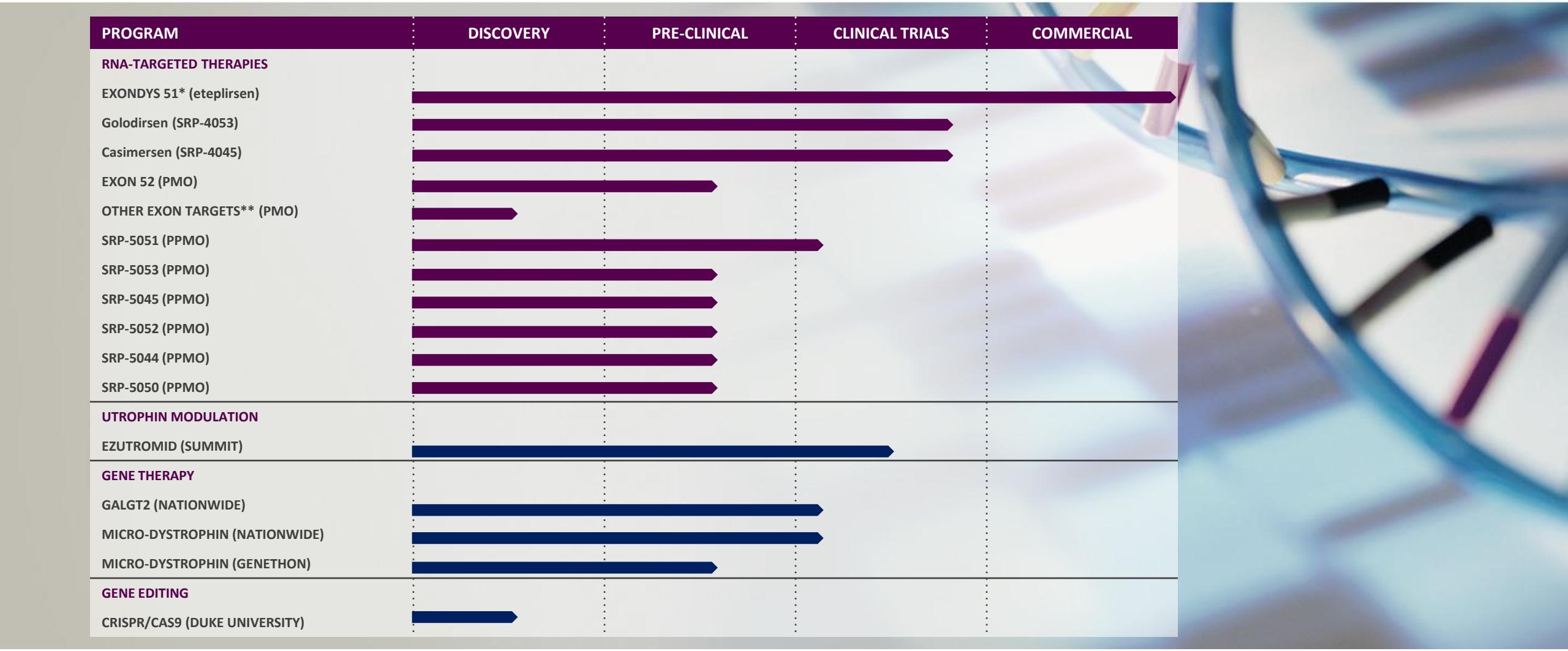
to accelerate R&D, expand talent base, and support partnering

2018: A YEAR OF TRANSFORMATION

KEY MILESTONES/INFLECTION POINTS



OUR PATH TO DEVELOPING TREATMENTS FOR ALL ELIGIBLE INDIVIDUALS WITH DMD

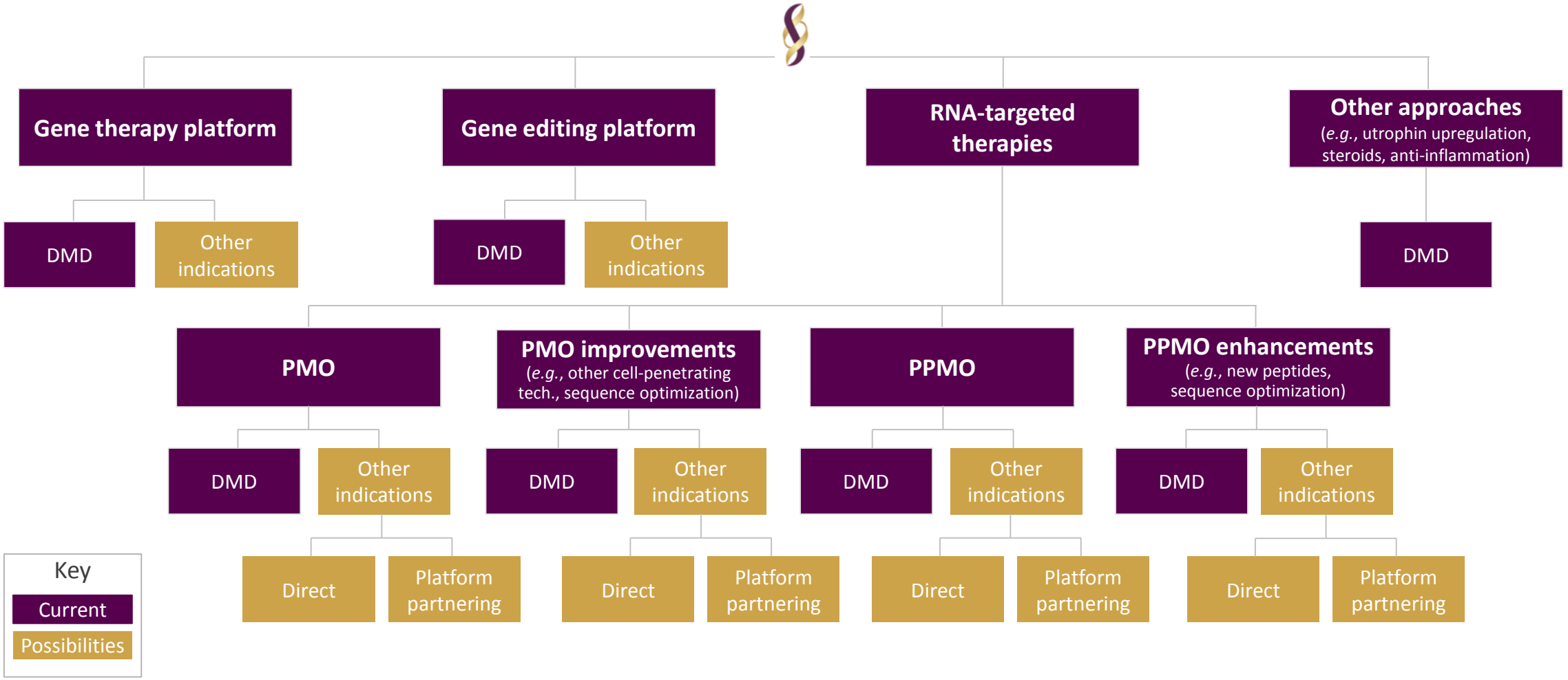


Internal
External Collaborations

*EXONDYS 51 received accelerated approval in the U.S., confirmatory studies required
**Other exon targets in development: 8, 35, 43, 44, 50, and 55

BUILDING A GLOBAL FRANCHISE IN NEUROMUSCULAR DISEASES

DEVELOPMENT STRATEGY



WHAT IS DMD?[1-2]

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

- Rare, progressive neuromuscular genetic disease that is 100 percent fatal
- Average lifespan of mid- to late-20s; typical diagnosis occurs between ages 4-5
- Caused by gene mutation that encodes dystrophin, a protein that exists in infinitesimally small amounts in the body (0.002 percent of muscle), but plays a key structural role in muscle fiber production
- Even small amounts of dystrophin production have shown significant benefits (*e.g.* Becker Muscular Dystrophy and exon 44 amenable individuals)

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

EXPERTS IN RNA-TARGETED THERAPIES

CORE TECHNOLOGY PLATFORM

USING PRECISION RNA SPLICING TO CORRECT GENETIC DEFECTS

Our RNA platform results in genetic medicine precisely engineered to induce pre-mRNA splicing, which is designed to transform mRNA and produce a truncated but functional protein

Sarepta Therapeutics is currently advancing two RNA-targeted technologies

Phosphorodiamidate Morpholino Oligomer (PMO)

- Synthetically designed structures modeled after the natural framework of RNA
- Near-term opportunity with potential to treat 29 percent of individuals with DMD by 2019

Peptide Phosphorodiamidate Morpholino Oligomer (PPMO)

- Designed to enhance tissue targeting, intracellular delivery, target selectivity, and potency
- Potentially transformative approach to slowing disease progression in DMD with application in other therapeutic areas

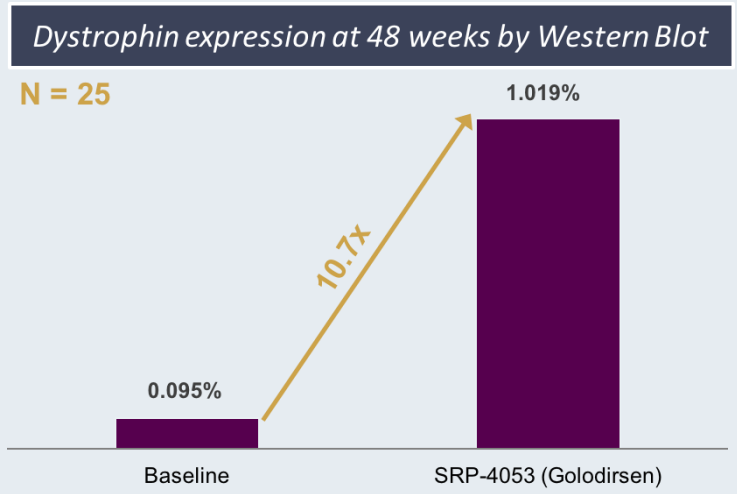
PMO PROGRAMS: SEVERAL POTENTIAL NEAR-TERM CATALYSTS

APPROVED THERAPY AND LATE-STAGE CANDIDATES TARGET 29 PERCENT OF INDIVIDUALS WITH DMD

EXONDYS 51™ (eteplirsen) Injection

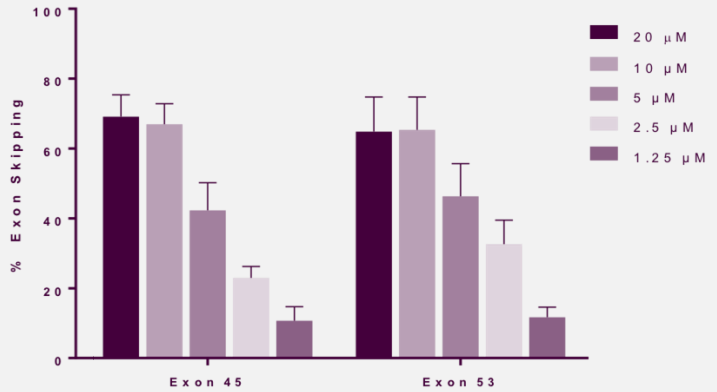
- ✓ Strong U.S. launch
- ✓ Managed Access Program opened or opening in 33 countries
- ✓ Marketing Authorization Application (MAA) currently under review in Europe

Golodirsen (SRP-4053)



- ✓ Meeting with U.S. FDA in Q1'18 to determine regulatory pathway

Casimersen (SRP-4045)



- ✓ Exon-skipping efficiency in pre-clinical models comparable to golodirsen
- ✓ Being evaluated in ongoing Phase 3 ESSENCE clinical trial

Targets 13% of the DMD population

Potential to treat an additional 16% of the DMD population

PPMO: A TRANSFORMATIVE APPROACH TO TREATING DMD

PPMO

SRP-5051: first PPMO candidate in the clinic

STATUS

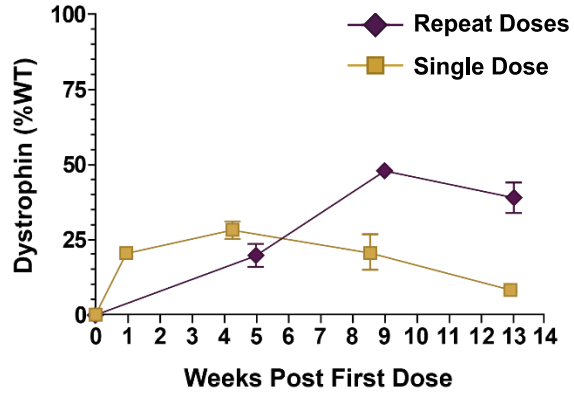
- Phase 1/2a clinical trial initiated in November 2017
- Multi-center, double blind, placebo-controlled, multi-dose efficacy portion of study to initiate by mid-2018 or as soon as a therapeutic dose has been identified

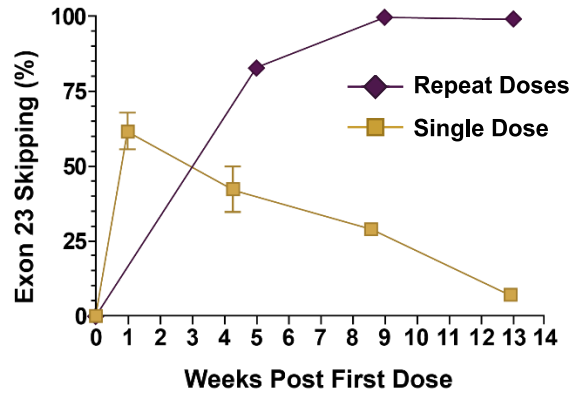
- Superior delivery vehicle
- Induces significant increases in dystrophin levels
- Potentially less frequent dosing in the clinic
- Achieves delivery in the three major muscle groups affected in DMD

Sources: Internally generated data

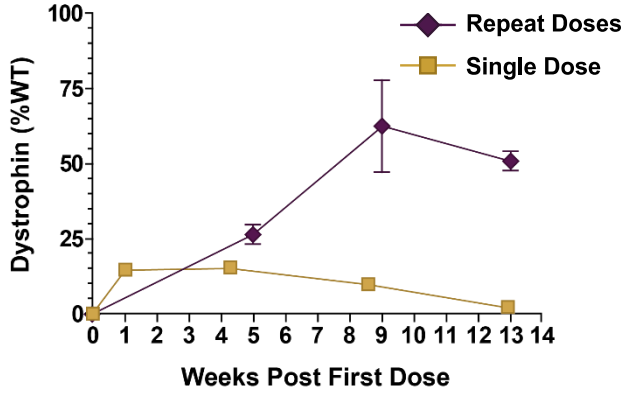
REPEAT PPMO DOSES INCREASED AND SUSTAINED DYSTROPHIN PRODUCTION *IN VIVO*

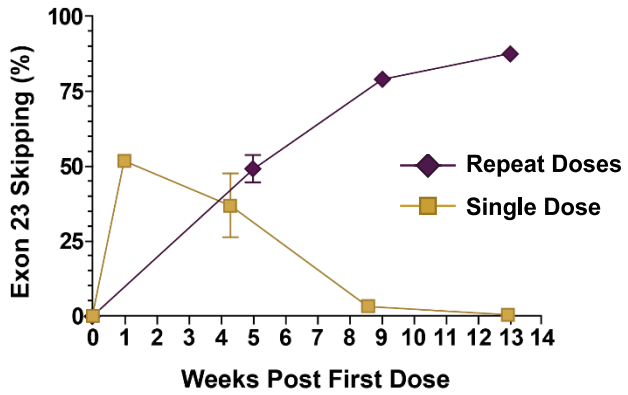
Quadriceps



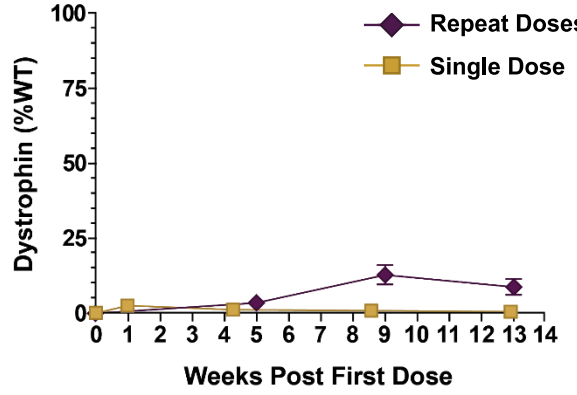


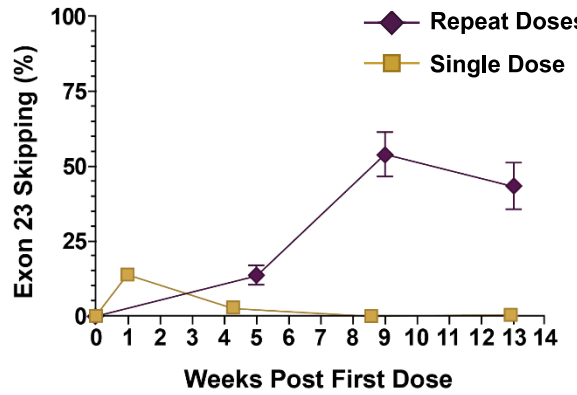
Diaphragm





Heart

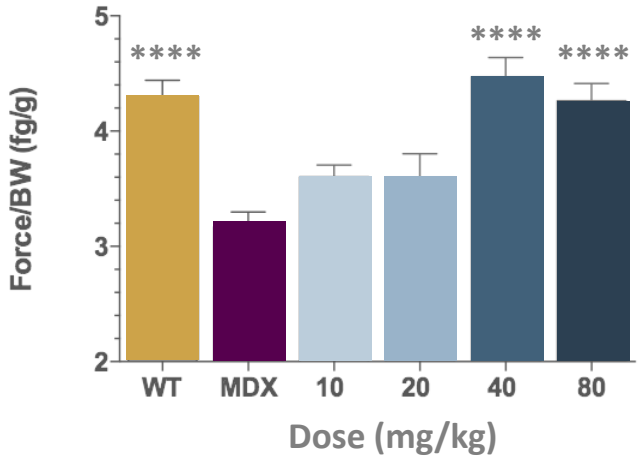




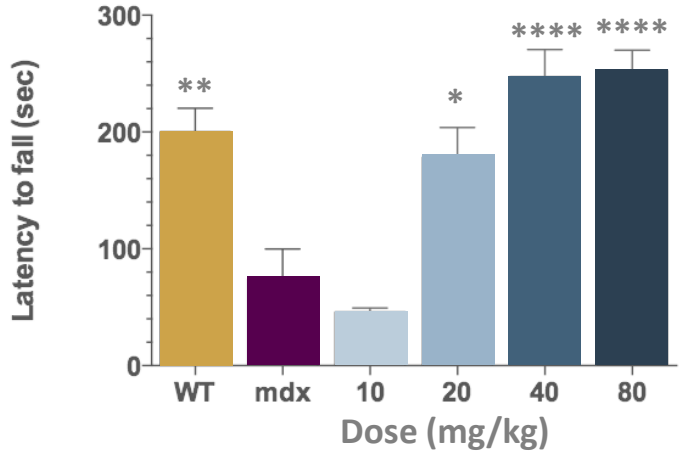
mdx (DMD) mice were treated with a three monthly IV doses of PPMO @ 40 mg/kg; the single dose PPMO cohort was included as a comparator

PPMO TREATMENT IMPROVED MUSCLE FUNCTION IN *MDX* MICE

Grip Strength (8 weeks)

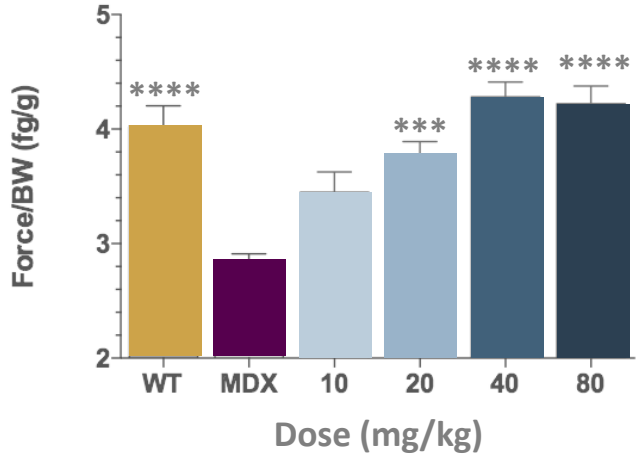


Rotarod (9 weeks)

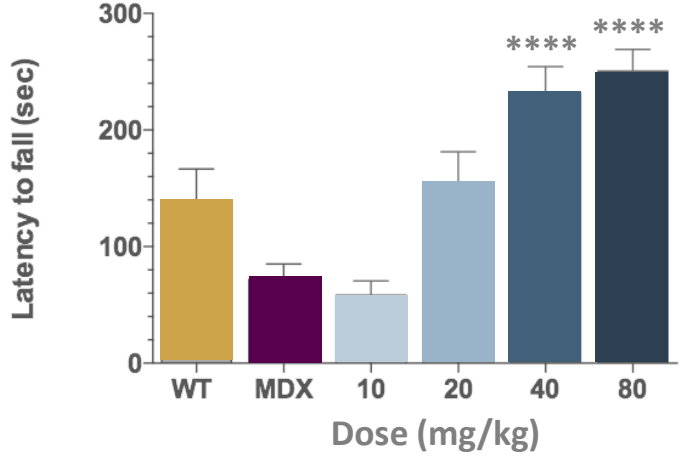


Grip Strength (10 weeks)

Mean +/- SE



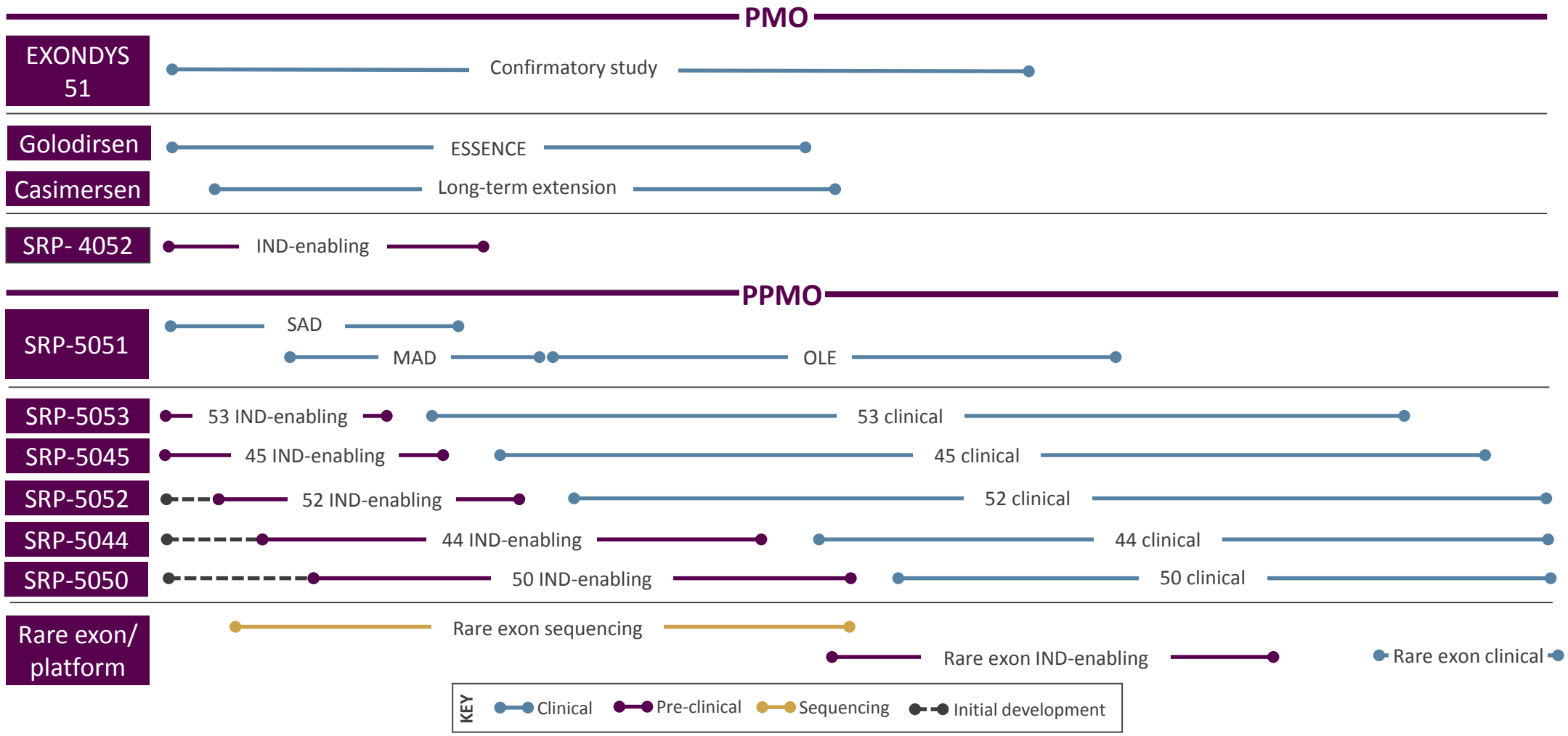
Rotarod (11 weeks)



- *mdx* (DMD) mice at 7 weeks of age were treated with a single IV dose of saline or PPMO @ 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 8 weeks (1 week post-injection, pi) and 10 weeks (3 weeks pi) of age, and for rotarod performance at 9 weeks (2 weeks pi) and 11 weeks (4 weeks pi) of age (n=10 per group)
- Graphs are mean +/- SE; Statistics performed was the **One Way Anova Tukey Multiple Comparison** Test and the significant values shown are versus mdx saline (*p<0.05, **p<0.01, ***p<0.001, ****p<0.0001)

RNA-TARGETED THERAPY FRANCHISE DEVELOPMENT STRATEGY*

POTENTIAL FOR UP TO SEVEN APPROVED THERAPIES IN THE U.S. BY 2022



**timeline not to scale*

NEXT-GENERATION APPROACHES

ONGOING GENE THERAPY AND EDITING PROGRAMS

LEADERS IN GENE THERAPY AND EDITING FOR DMD

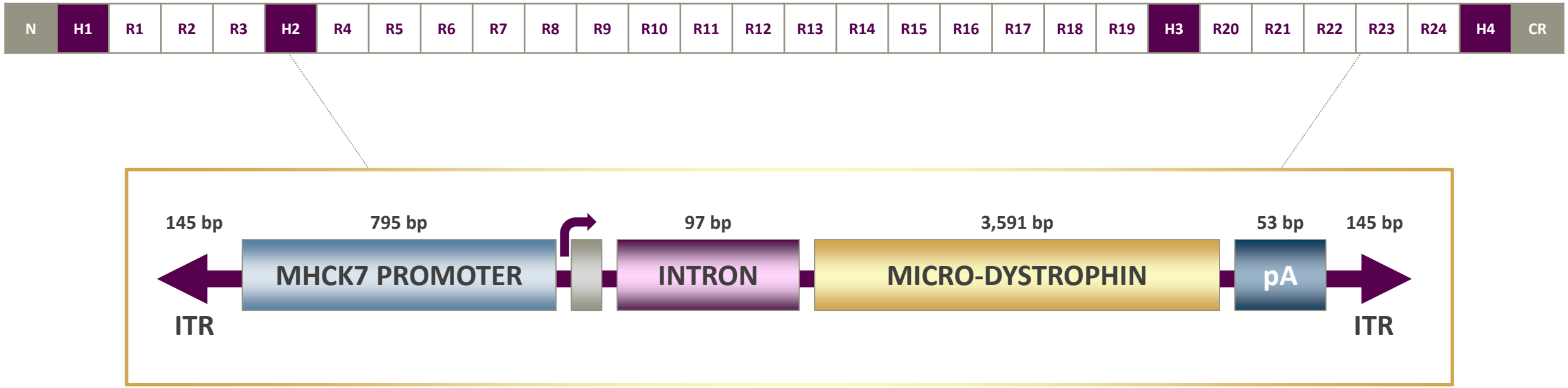
POTENTIAL TO ADDRESS ALL INDIVIDUALS WITH DMD

PARTNER	Nationwide Children's Hospital	Genethon	Nationwide Children's Hospital	Duke University
ANNOUNCED	January 2017	June 2017	January 2017	October 2017
MODALITY	Micro-Dystrophin Gene Therapy	Micro-Dystrophin Gene Therapy	GALGT2 Gene Therapy	CRISPR/Cas9 Gene Editing
TYPE	Research and option agreement	Research and option agreement	License agreement	Research and option agreement
STATUS	Phase 1/2 underway	Manufacturing scale-up underway	Phase 1/2 underway	Technology optimization

MICRO-DYSTROPHIN GENE THERAPY OVERVIEW

PHASE 1/2A CLINICAL TRIAL UNDERWAY; INTERIM SAFETY AND EFFICACY DATA EXPECTED IN 2H'18

Program developed and led by gene therapy pioneers Jerry Mendell, M.D. and Louise Rodino-Klapac, Ph.D.



Uses an adeno-associated virus vector to deliver a shortened version of the dystrophin gene to replace the missing protein in individuals with DMD

THE PROMISE OF MICRO-DYSTROPHIN GENE THERAPY IN DMD

GENETHON DATA PUBLISHED IN *NATURE COMMUNICATIONS*

- Study conducted in 12 dogs naturally affected by DMD and treated with Genethon's micro-dystrophin gene therapy
- At two-year follow-up, muscle function was significantly restored and clinical symptoms had stabilized
- Dystrophin expression had returned to a high level in the high-dose group
- No immunosuppressive treatment was administered beforehand, and no side-effects were observed

Video courtesy of:

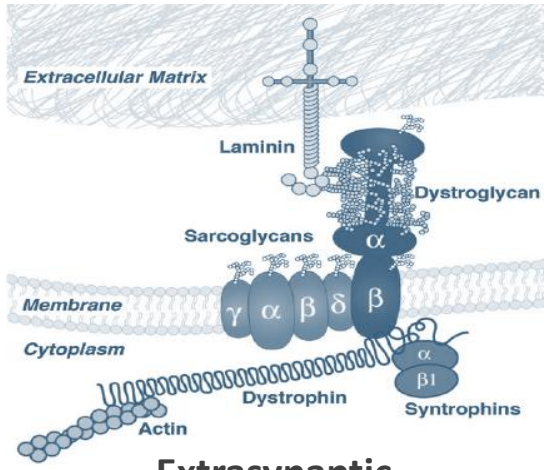
GENETHON
CURE THROUGH INNOVATION

GALGT2 GENE THERAPY OVERVIEW

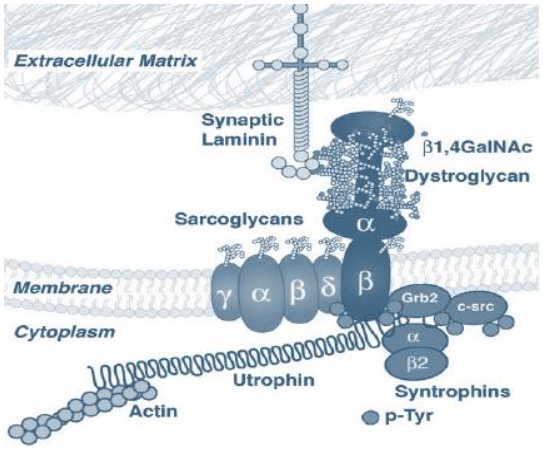
POTENTIAL TO RESTORE MUSCLE FUNCTION DESPITE THE ABSENCE OF THE DYSTROPHIN PROTEIN

TARGET | Targets the dystroglycan complex to enhance utrophin expression—a largely homologous protein to dystrophin—and preserve muscle function regardless of underlying genetic mutation

Normal Muscle

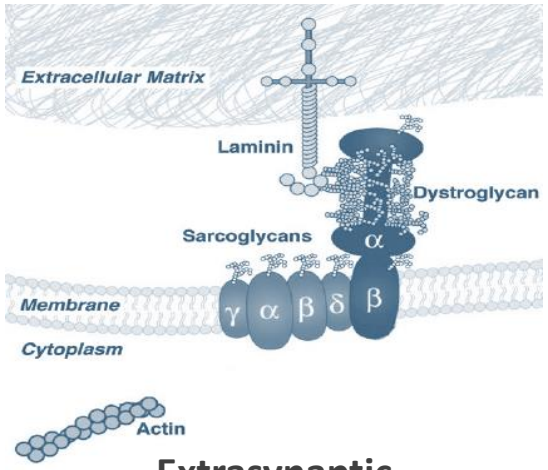


Extrasynaptic



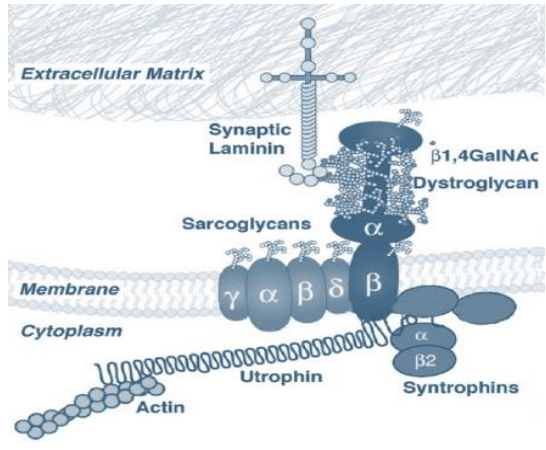
Synaptic
Galgt2

DMD Muscle



Extrasynaptic
mdx

GALGT2-Treated DMD Muscle



Extrasynaptic
mdx
Galgt2 Transgenic

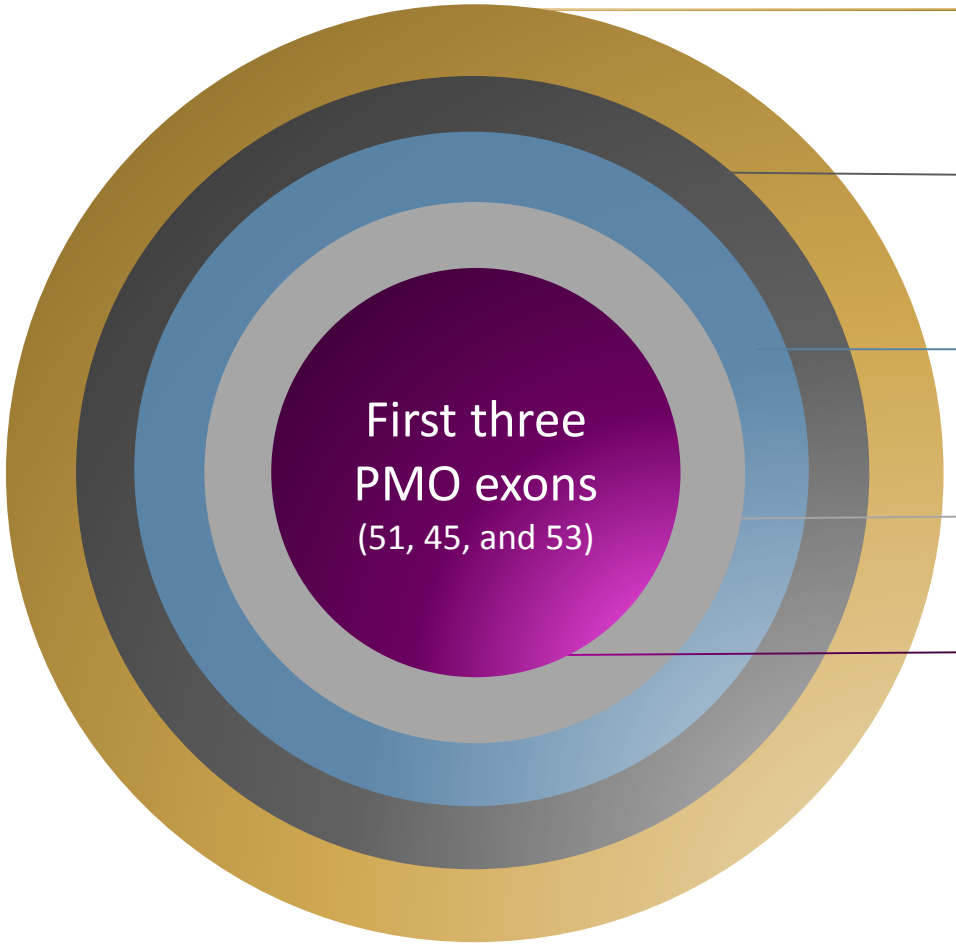
STATUS

- Dose escalation trial beginning with the minimal efficacious dose as determined by pre-clinical studies
- Interim read-out assessing safety and efficacy signals in 2H'18

Adapted from Flanigan 2014 PPMD presentation

WELL-POSITIONED FOR FUTURE SUCCESS

OUR BASE CASE REPRESENTS A SIGNIFICANT OPPORTUNITY



OTHER DISEASES — Expansion into other therapeutic areas

GENE THERAPY — Potential to address 100 percent of eligible individuals with DMD

PPMO — A potentially transformative approach to slowing disease progression in DMD

OTHER PMO EXONS — Other PMO exon targets being developed

Base case: >$2.0B

potential peak sales for late-stage PMO therapies

EXONDYS 51: STRONG QUARTERLY GROWTH

Strong uptake through first five quarters post-launch

Key drivers of success:

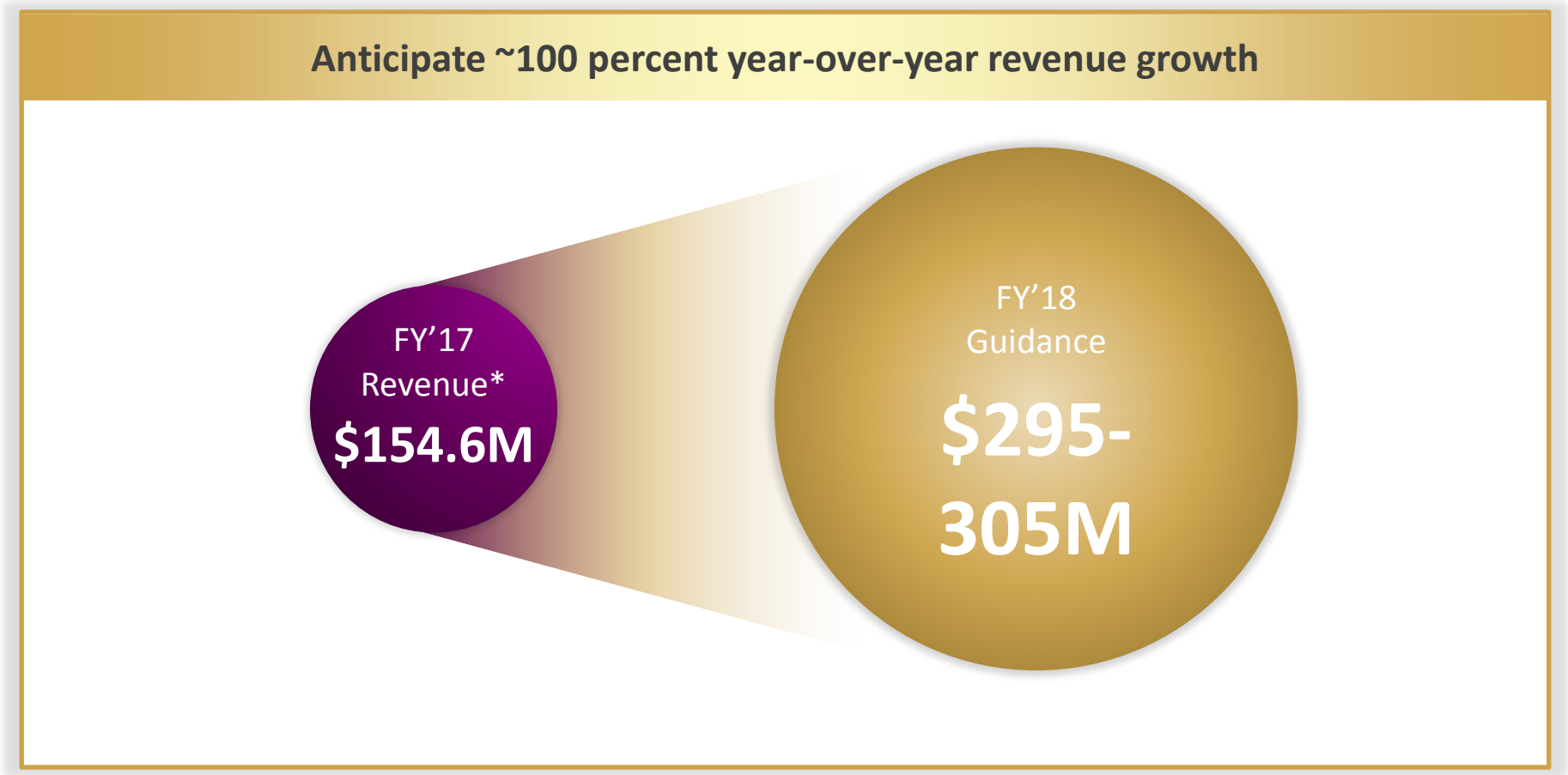
Major DMD centers continue to submit START forms

Reimbursement wins in 2017

More eligible individuals getting on therapy

***Unaudited**

EXONDYS 51: STRONG GROWTH IN 2018



*Unaudited