SAREPTA THERAPEUTICS

EXON SKIPPING: WHERE ARE WE NOW?

ACTION DUCHENNE INTERNATIONAL CONFERENCE

NOVEMBER 6, 2015

FORWARD LOOKING STATEMENTS

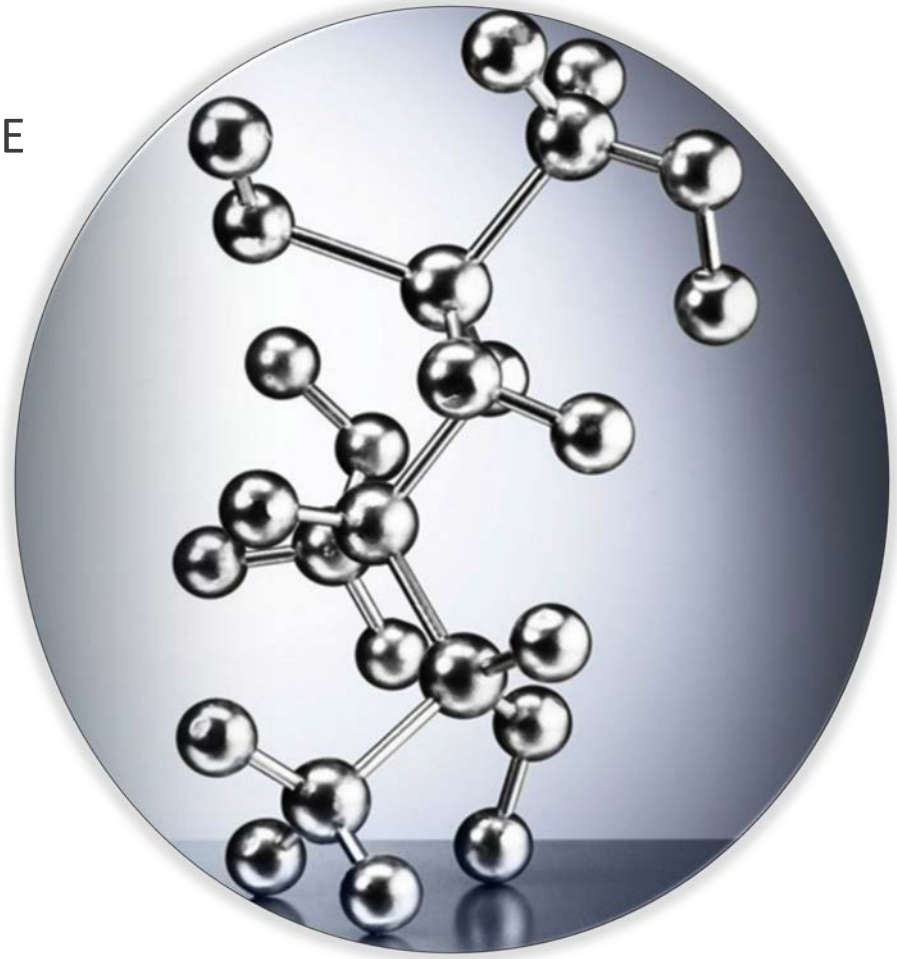
This presentation contains forward-looking statements. These forward-looking statements generally can be identified by the use of words such as "believes or belief," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," "advance" and similar expressions. These forward-looking statements include statements about our ability to accelerate the development of future candidates and provide patients access; our ability to develop treatments for each exon, alone or in combination with other drugs; the potential and timing of an NDA submission for eteplirsen and other candidates in the treatment of DMD; the planned results and submission of additional data, analysis and other information with the eteplirsen NDA; the regulatory path forward for eteplirsen and other candidates; the timing and design of and our ability to initiate and complete additional studies for eteplirsen and other candidates; the potential filing and acceptance of an NDA for eteplirsen and other candidates by the FDA and EMA; the potential and timing of an advisory committee for the eteplirsen NDA; the potential and timing for regulatory approval of eteplirsen, including on an accelerated pathway; and our commitment to the DMD Community

Each forward-looking statement contained in this presentation is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statement. Applicable risks and uncertainties include, among others: we may not be able to comply with all FDA requests and requirements; the FDA may determine that substantial additional data is required for accelerated or other approval of eteplirsen or that our NDA submission for eteplirsen does not qualify for filing, even with additional data and information; the results of our ongoing and new clinical trials may not be positive; there may be delays in timelines relating to an NDA submission, initiating clinical trials, or making a product commercially available for regulatory or internal reasons; we may not be able to manufacture sufficient supply for clinical trials or commercialization; Agency or court decisions with respect to our patents may negatively impact our business; and those identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2014 filed with the Securities and Exchange Commission (SEC), and Sarepta's other filings with the SEC.

Any of the foregoing risks could materially and adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

OVERVIEW

- SAREPTA THERAPEUTICS – WHO WE ARE
- PMO CHEMISTRY
- EXON-SKIPPING
- DYSTROPHIN
- CLINICAL DEVELOPMENT
- REGULATORY UPDATE
- OUR VISION



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OUR COMMITMENT

To translate scientific breakthroughs into meaningful advances in treatment for patients.

- U.S. based (~250 employees):
  - Headquarters: Cambridge, Massachusetts
  - Research Facility: Corvallis, Oregon
  - Manufacturing Facility: Andover, Massachusetts
- Leadership team comprised of industry professionals with extensive Research & Development and Commercial experience

Sarepta offices | Cambridge, MA USA

PHOSPHORODIAMIDATE MORPHOLINO OLIGOMER (PMO) CHEMISTRY

VERSATILITY

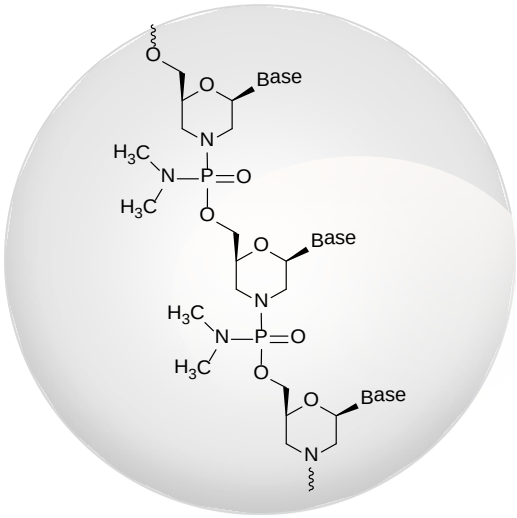
PMOs are highly adaptable molecules and, with minor modifications, can potentially be rapidly designed to target specific tissues, genetic sequences, or pathogens.

SPECIFICITY

PMOs are charge neutral which may limit interactions with proteins in the body other than the target RNA.*

STABILITY

PMOs are highly resistant to degradation by enzymes, potentially enabling drug activity.*



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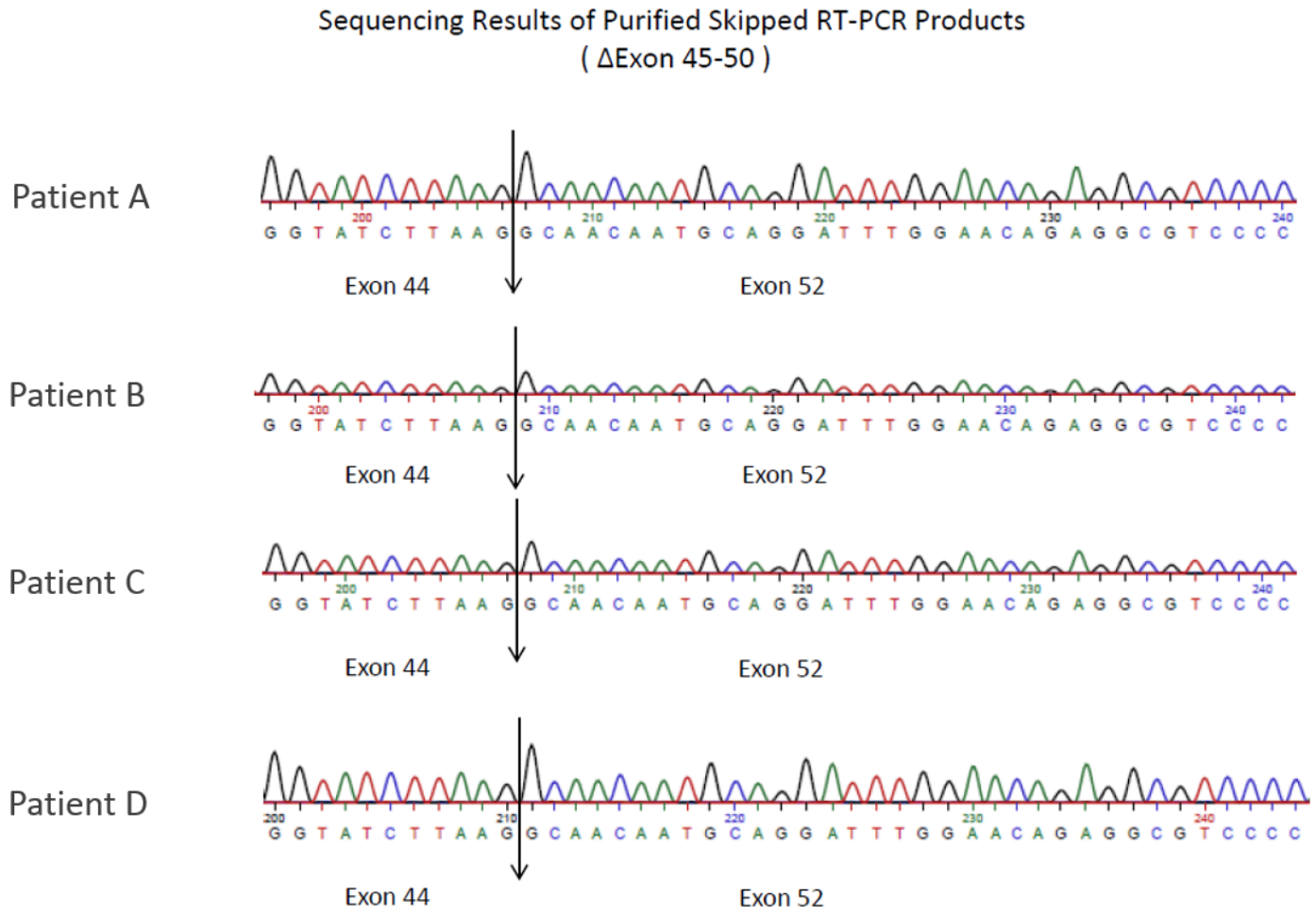
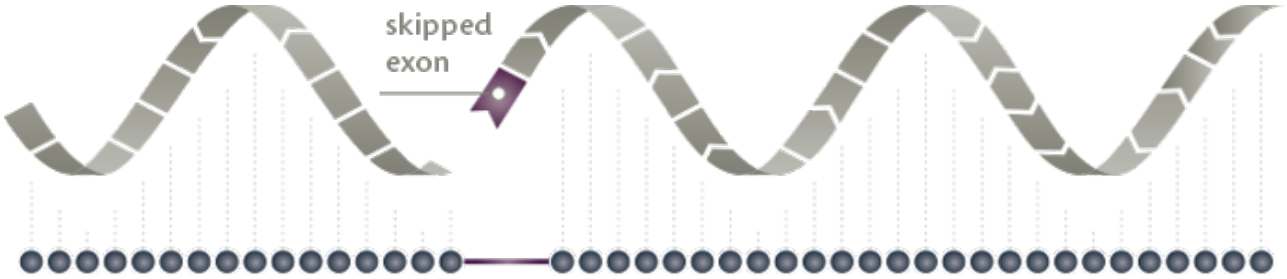
*Hudziak et al., Resistance of morpholino phosphorodiamidate oligomers to enzymatic degradation. Antisense and Nucleic Acid Drug Development. 1996; 6(4) 267-72

EXON SKIPPING

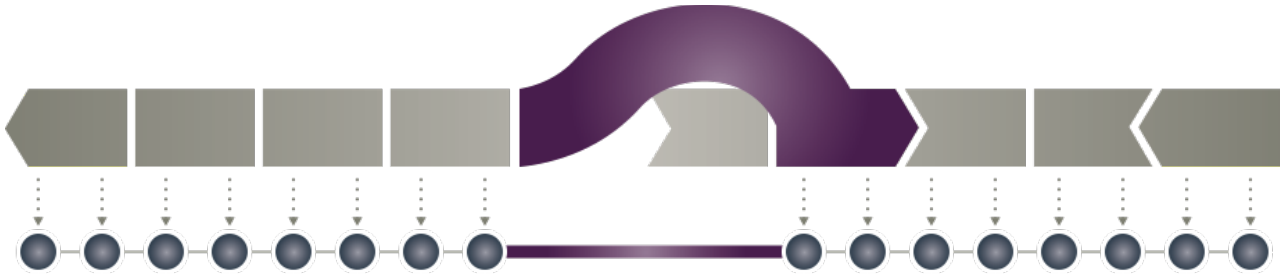
CONFIRMS MECHANISM OF ACTION IN PATIENTS TREATED & EVALUATED TO DATE



DYSTROPHIN PRODUCTION



Our investigational therapies for DMD are designed to skip an exon in the dystrophin pre-mRNA to enable the synthesis of a functional shorter form of the dystrophin protein.



Exon skipping is a potential treatment approach to correct for specific mutations and restore production of dystrophin protein.

HOW DO YOU MEASURE DYSTROPHIN PRODUCTION?

Each Method of Dystrophin Analysis has a Different Functional Use					
Measurement	Proof of exon-skipping	Sarcolemma protein	Total protein	Semi-Quantitative	Validated
RT-PCR	✓				
IHC Dystrophin Intensity		✓		✓	✓
IHC % Dystrophin Positive Fibers		✓		✓	✓
Western Blot			✓	✓	✓

CLINICAL DEVELOPMENT

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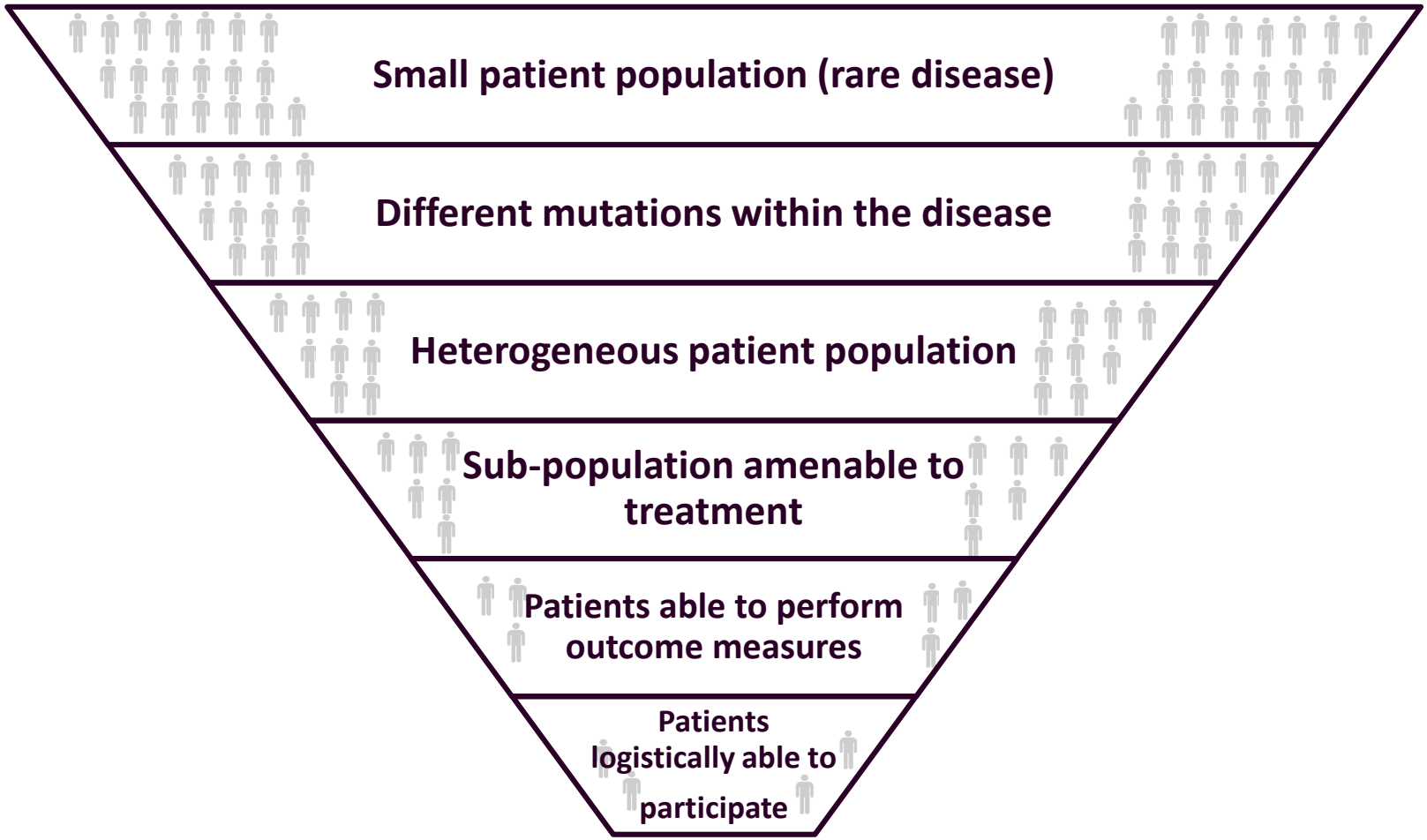
CHALLENGES FACING DUCHENNE DRUG DEVELOPMENT

THE IMPACT OF PERSONALIZED MEDICINE IN RARE DISEASE



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MECHANISMS TO OVERCOME THESE CHALLENGES

INCREASE SPEED & EFFICIENCY

Preclinical

- Biomarker development and validation
- Bridging of pharmacokinetics and toxicology

Clinical

- Innovative clinical trial design and analysis
- Master protocols
- Surrogate endpoints
- Patient reported outcomes (PROs)
- Natural history
- Untreated arm vs. placebo

Regulatory review and approval

- Accelerated Approval
- Fast Track
- Priority Review

CLINICAL DEVELOPMENT UPDATE

SAREPTA'S DEVELOPMENT PATHWAY IS PURPOSEFUL

STUDY	EXON TARGET	KEY FEATURE	STATUS
4658-202 (US)	Exon 51	>3.5 years of efficacy and safety data[1]	Enrollment complete
4658-301 *PROMOVI* (US)	Exon 51	Untreated arm to build natural history data for genotypes	Enrollment on schedule
4658-204 (US)	Exon 51	Broaden patient population (ages 7-21; limited/no ambulation)	Enrollment complete
4658-203 (US)	Exon 51	Broaden patient population (ages 4-6) Explore less invasive biomarker (MRI) Untreated arm to build natural history data	First Patient Dosed July 2015
4045-301 *ESSENCE* (US/EU)	Exon 45/53	Master protocol to increase efficiencies in study Broaden patient population (genotype) Global Study	Initiation planned
4045-101 (US)	Exon 45	Broaden patient population (genotype; non-ambulatory) Dose escalation study could potentially expedite access to patients globally	First Patient Dosed October 2015
4053-101 (EU)	Exon 53	Broaden patient population (genotype) Untreated arm to build natural history data	Enrolling part II

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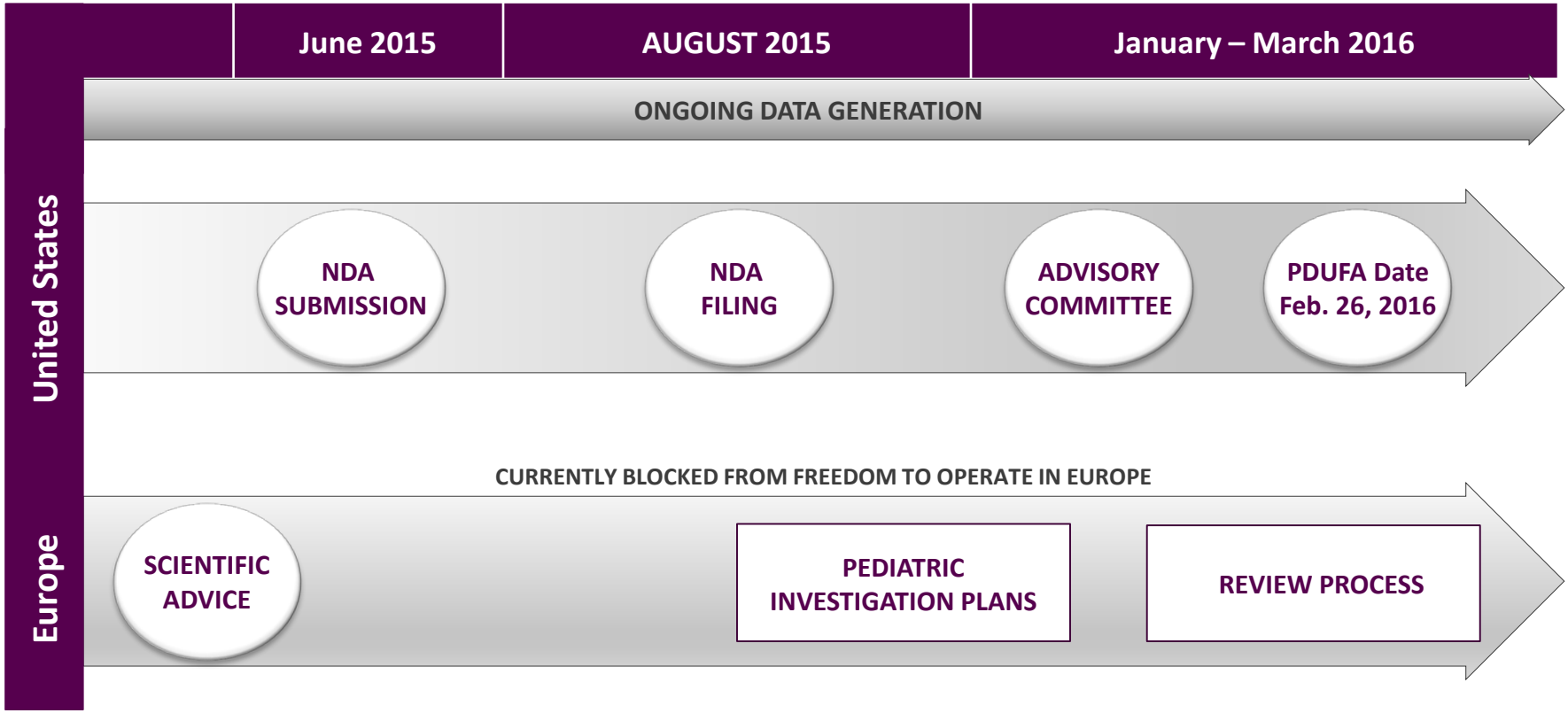
[1]212 weeks in extension phase for a total 201/202 duration of 240 weeks

KEY ETEPLIRSEN REGULATORY ACTIVITIES

SAREPTA'S TENTATIVE ADVISORY COMMITTEE DATE: JANUARY 22, 2015 | PDUFA DATE: FEBRUARY 26, 2016



EMA regulatory timeline is subject to future discussions with the EMA.

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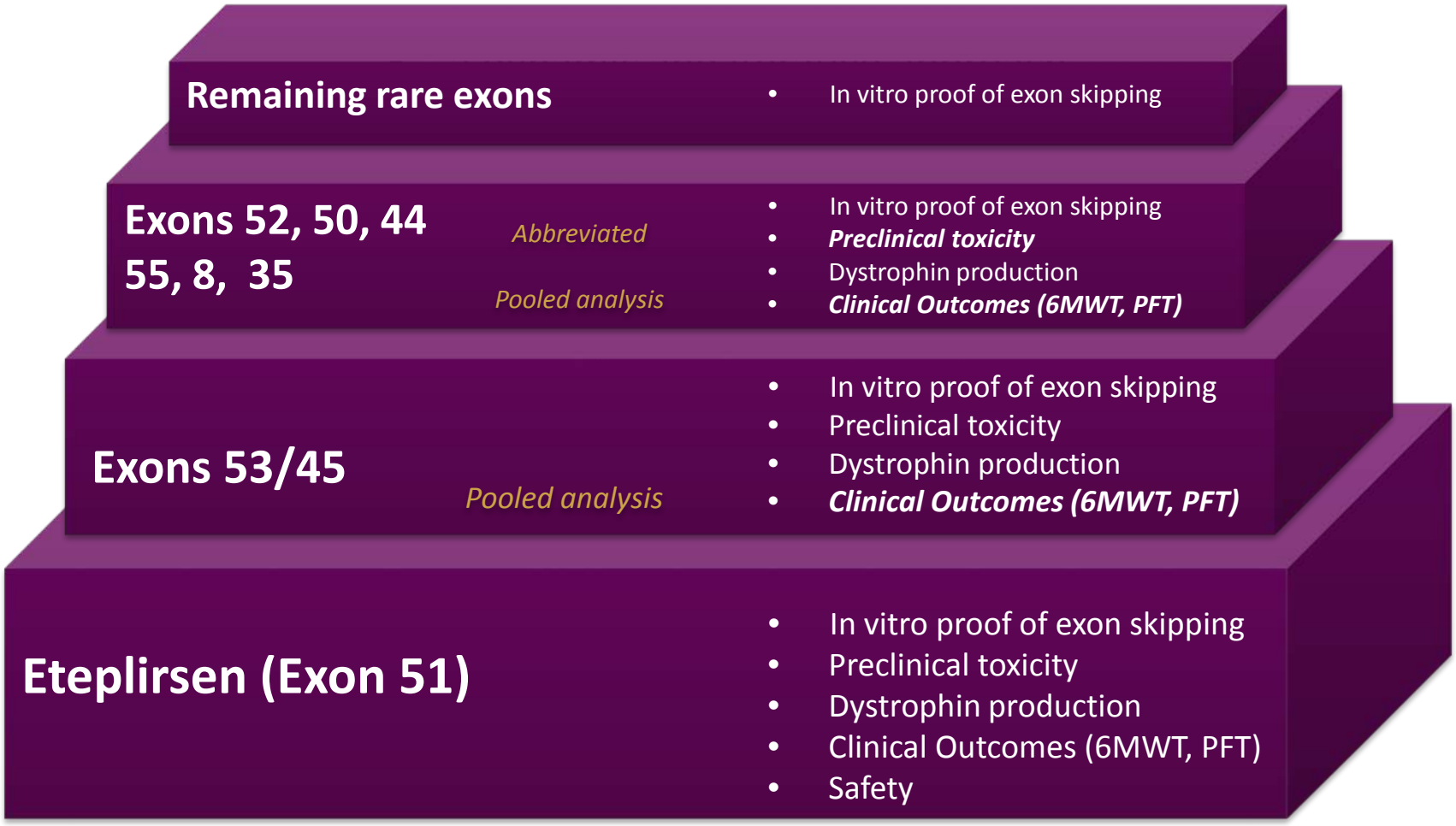
BUILDING THE FOUNDATION TO ACCELERATE DEVELOPMENT



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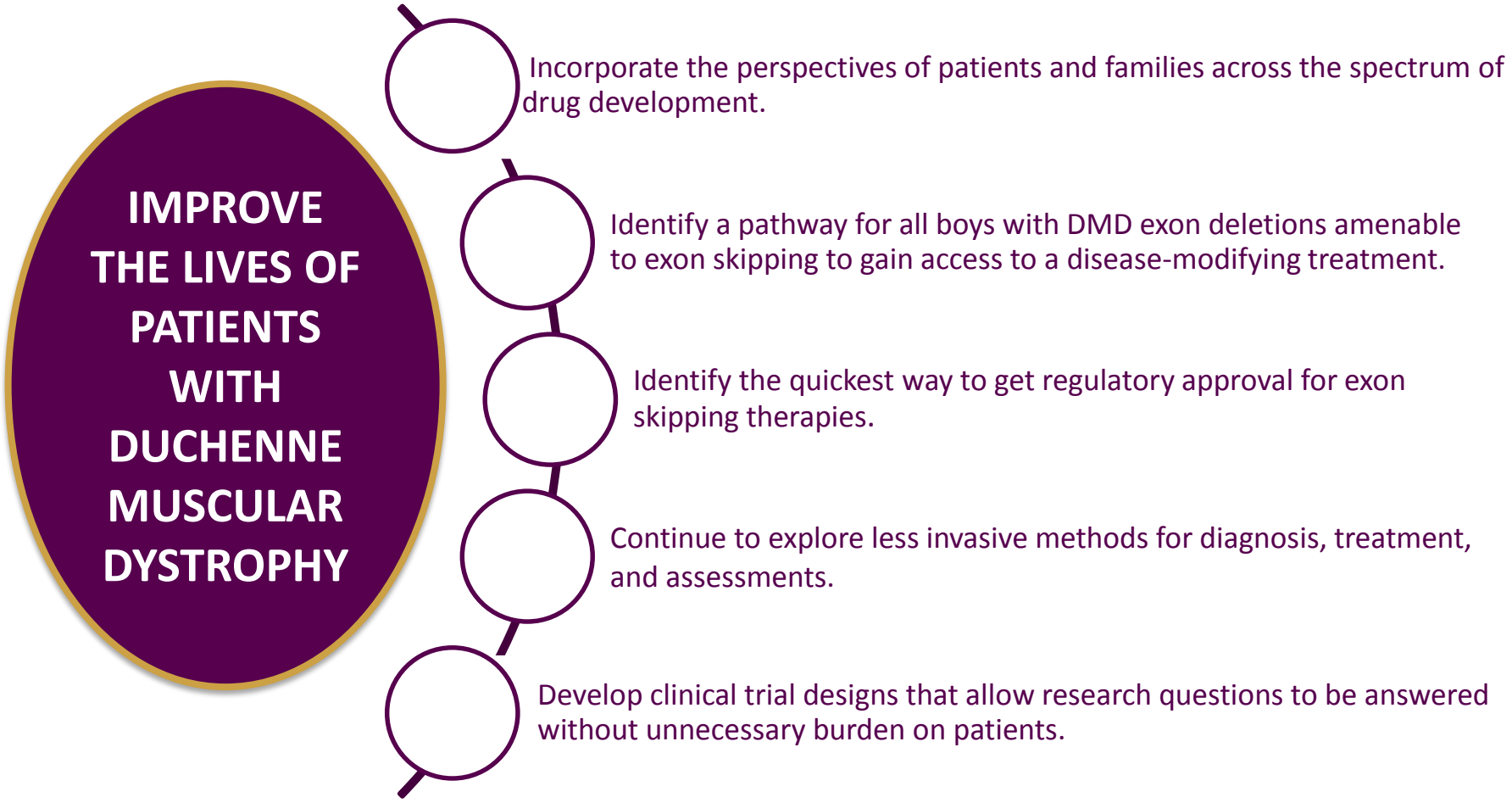
OUR VISION


IMPROVE THE LIVES OF PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY
Incorporate the perspectives of patients and families across the spectrum of drug development.
Identify a pathway for all boys with DMD exon deletions amenable to exon skipping to gain access to a disease-modifying treatment.
Identify the quickest way to get regulatory approval for exon skipping therapies.
Continue to explore less invasive methods for diagnosis, treatment, and assessments.
Develop clinical trial designs that allow research questions to be answered without unnecessary burden on patients.

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THANK YOU