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## 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 pipeline, programs and technologies and their respective potential benefits, including the benefits of MHCK7, AAVrh74, SR2 and SR3; the expectation to commercialize golodirsen in 2019 and to have 3 RNA-therapies on the market by 2020, serving approximately 30% of the DMD community; our plan to take scientific advice with EMA in connection with eteplirsen; our intention to play a central role in transforming the health care ecosystem; our opportunities with genetic medicine and in particular gene therapy; our goal to build a gene therapy engine; our expected path forward in connection with our micro-dystrophin program; the potential of gene therapy to upend the traditional R&D model and our intention to play a central role in that upending; the potential of gene therapy to have a higher probability of success from much earlier phase and shorter timelines; our goal to have more commercial supply in the next two years than all of the gene therapy manufacturing supply that exists in the world today; the estimated number of patients suffering from DMD, LGMD and CMT; Sarepta having the most robust precision genetic medicine pipeline in the world; our goal to continue to build our pipeline out and to have 40 programs; the potential of our programs to bring a better life to some portion of the patients living with rare diseases covered by our programs; our milestones and expected timelines, including a target approval for golodirsen in Q3 of 2019, filing for casimersen by mid 2019 with a target approval by Q1 2020, commencing a confirmatory trial for our micro-dystrophin program later in 2019, having results in LGMD in Q1 2019, dosing later in 2019 in our program to treat CMT and commencing dosing in our program to treat MPS3A in Q1 2019; 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: the audit of our financial statements for the year ended December 31, 2018 is ongoing and could result in changes to the information; we may not be able to meet expectations with respect to EXONDYS 51 sales, 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; our data for golodirsen, casimersen and/or our micro-dystrophin program may not be sufficient for obtaining regulatory approval; our product candidates, including those with strategic partners, may not result in viable treatments suitable for commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; if the actual number of patients suffering from DMD, LGMD, and/or CMT is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; our dependence on our manufacturers to fulfill our needs for our clinical trials and commercial supply, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of products to successfully support various programs, including research and development and the potential commercialization of our gene therapy product candidates; we may not be able to successfully scale up manufacturing of our product candidates in sufficient quality and quantity or within sufficient timelines; 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 2017 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q 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.

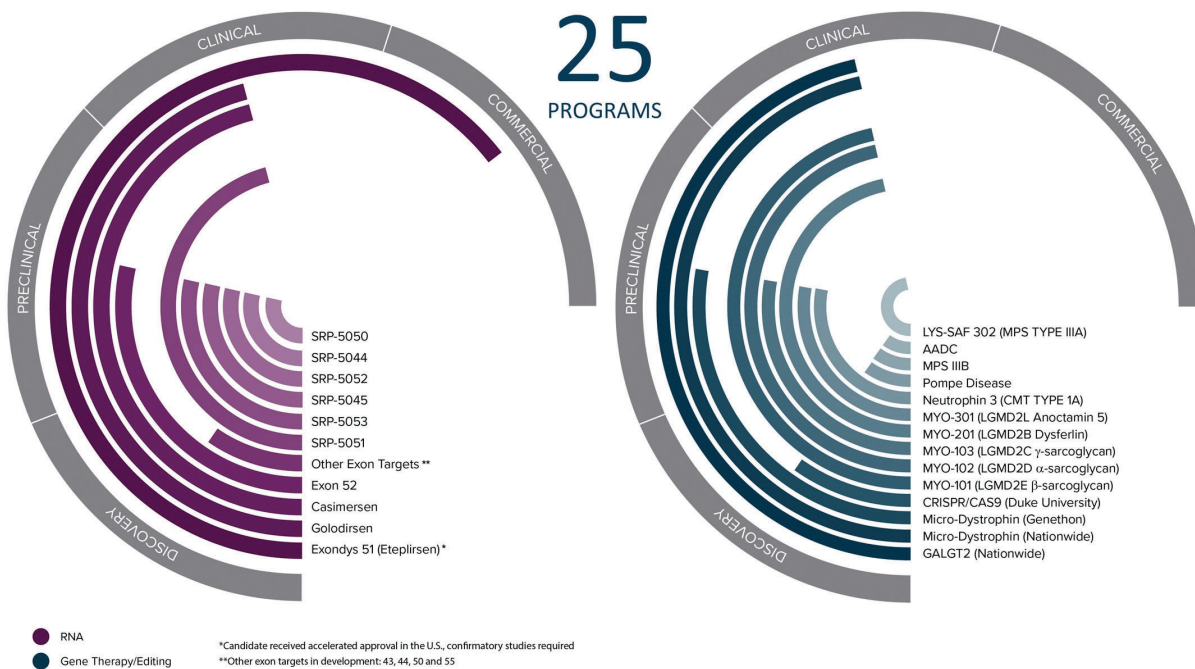
## OUR GOALS FOR 2018

- ✓ Build out our pipeline
- ✓ Ensure we have the resources to build for the future
- ✓ Clarify a pathway for our RNA technology
- ✓ Become one of the most important rare disease companies
- ✓ Have another strong year of commercial execution



# EXPANSION OF OUR RNA AND GENE THERAPY ENGINE

25  
PROGRAMS





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## RESOURCES AND TALENT\*

### CASH POSITION

>\$1.1B

### TALENT

500+  
PROFESSIONALS

2x PhDs

3x MDs

Leading gene therapy team

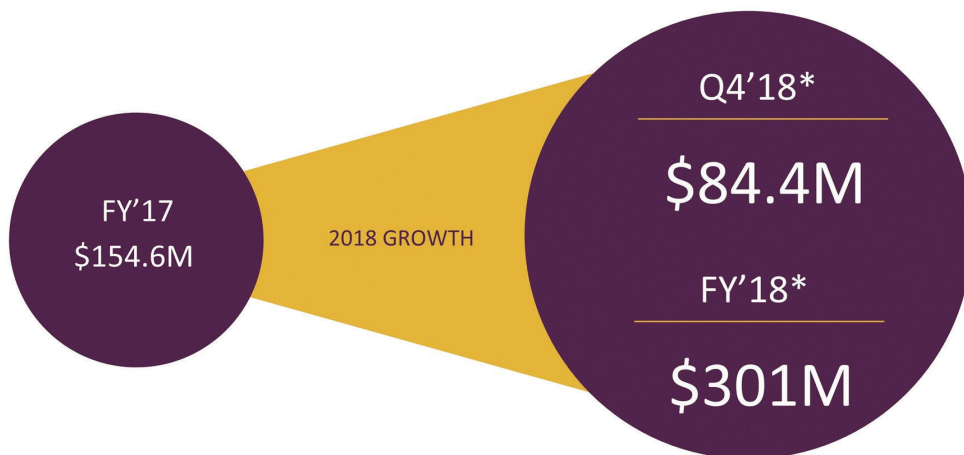
\*As of December 31, 2018; preliminary and unaudited results

## DEFINED RNA PATHWAY

If successful, we will have 3 RNA-therapies  
by 2020, serving ~30% of the DMD  
community.



# EXECUTION



\*Preliminary and unaudited results

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## TOP 5 RARE DISEASE COMPANIES\*



\*By Market Cap

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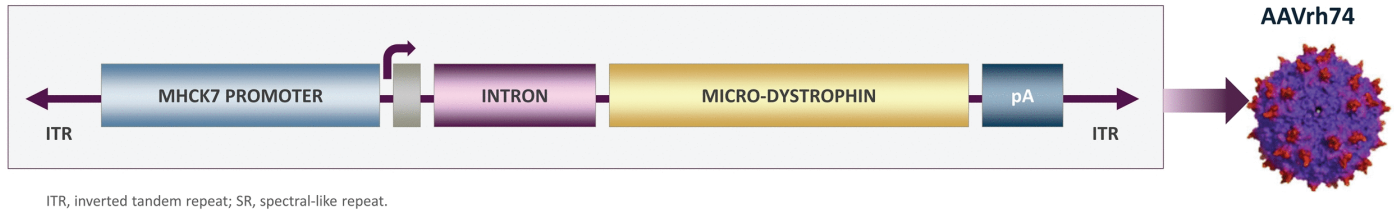


We are SAREPTA



## MICRO-DYSTROPHIN PROGRAM OVERVIEW

- **AAVrh74** provides broad distribution to **all** muscle types, including the heart and diaphragm
- **SR2** and **SR3** critical for force production
- **MHCK7** promoter enables robust dystrophin expression in heart and skeletal muscle



## UNPRECEDENTED EARLY STAGE RESULTS\*

**81%**

Robust expression of micro-dystrophin in muscle fibers

**96%**

Robust expression of micro-dystrophin measured by Western blot\*\* and signal intensity

Reduction of creatine kinase

**78%**

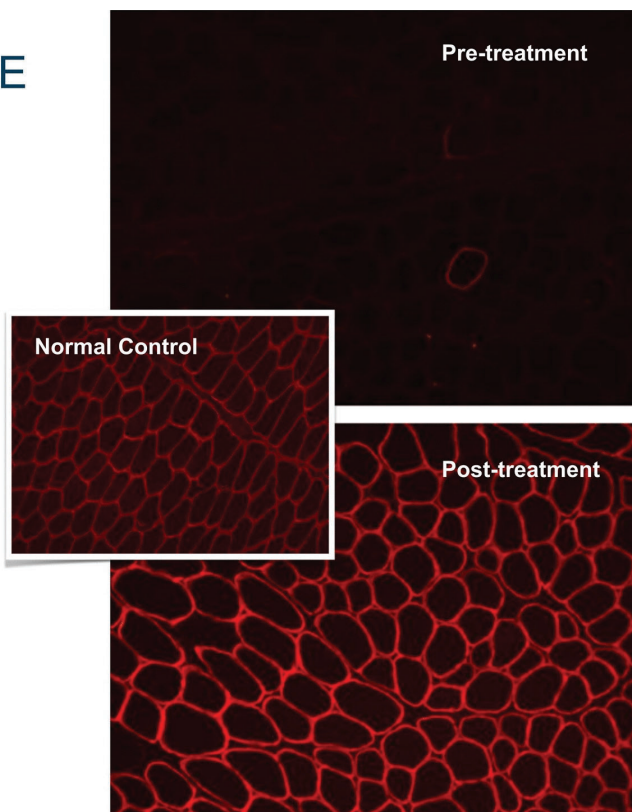
Improvements across all measured functions\*\*\*

Well-tolerated

\* Data from the 4 patients dosed in Study NCT03375164

\*\*NCH Western blot method

\*\*\*North Star Ambulatory Assessment (NSAA), Time to Rise, 4 Stairs Up, and 100M



## THE PATH FORWARD

Met with  
FDA in

Q4 2018

Current Trial

24 Patients  
Placebo controlled

Started  
Dosing

2 Patients

Study  
Goal

Functional Benefit  
of Expression

Confirmatory Trial  
With Commercial  
Material

Goal to Commence in  
2019



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And our intent is to do this over and over again



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7,000 Rare Diseases

~80% are single gene mutations



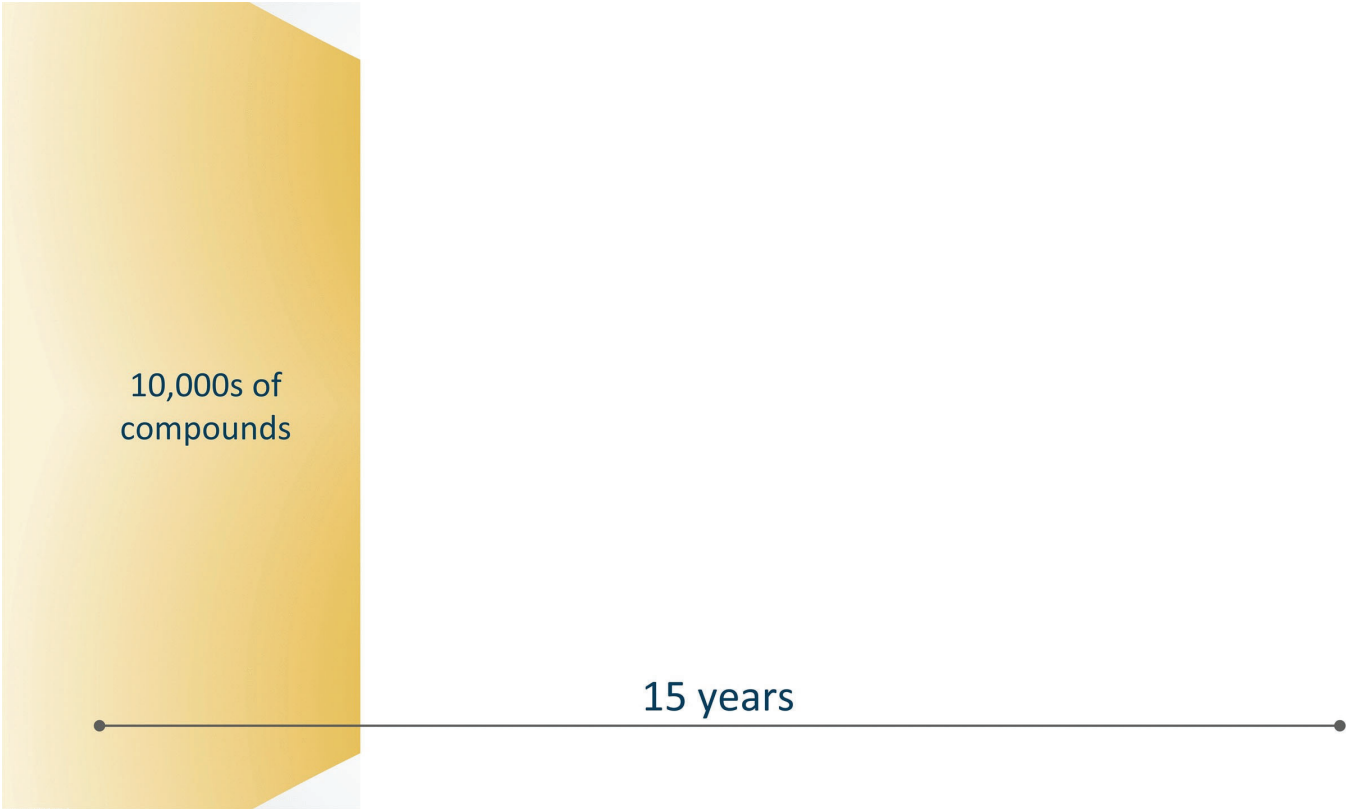
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● And Only 1 FDA Approved  
GENE THERAPY



**400M**  
**Patients**  
Impacted Globally

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10,000s of  
compounds

15 years

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## GENE THERAPY CAN UPEND THIS MODEL

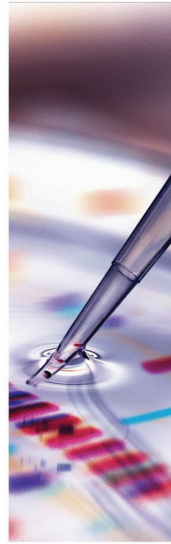


## AND WE WILL PLAY A ROLE IN UPENDING THE MODEL

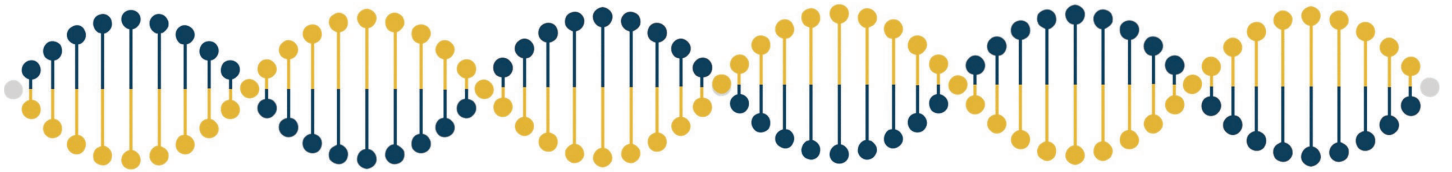
PROBABILITY OF SUCCESS

TIMELINES

MASSIVE OPPORTUNITY TO DO  
GOOD



# WE DON'T RELY ON SERENDIPITY WE ENGINEER SOLUTIONS







# OUR MANUFACTURING STRATEGY

The largest gene therapy capacity around the globe

Brammer

Paragon

Aldevron

In-House  
Capabilities

- Duchenne Muscular Dystrophy
- Limb-girdle Muscular Dystrophy
- Charcot-Marie-Tooth Disease
- Mucopolysaccharidosis
- Pompe Disease



MASSIVE OPPORTUNITY TO DO GOOD

## THE MOST ROBUST PRECISION GENETIC MEDICINE PIPELINE

TODAY

**25**  
Programs

GOAL

**40**  
Programs

EPIDEMIOLOGY\*

**>1.5M**  
patients with  
rare disease globally

\*Based on published epidemiology

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We are upending the model



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Because someday is too late



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PULLING  
TOMORROW  
INTO  
TODAY

