



Solid Biosciences

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**Parent
Project
Muscular
Dystrophy**

2019 ANNUAL
CONFERENCE

Forward-Looking Statements

This presentation includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, which involve a number of risks and uncertainties. These forward-looking statements include all matters that are not historical facts and, without limiting the foregoing, can be identified by the use of forward-looking terminology, including the terms “believe,” “estimate,” “project,” “anticipate,” “expect,” “seek,” “predict,” “continue,” “possible,” “intend,” “may,” “might,” “will,” “could,” “would” or “should” or, in each case, their negative, or other variations or comparable terminology. They appear in a number of places throughout this presentation and include statements regarding our intentions, beliefs or current expectations concerning, among other things, our product candidates, research and development and clinical trial plans, manufacturing plans, commercialization objectives, prospects, strategies, the industry in which we operate and potential collaborations. We derive many of our forward-looking statements from our operating budgets and forecasts, which are based upon many detailed assumptions. While we believe that our assumptions are reasonable, we caution that it is very difficult to predict the impact of known factors, and, of course, it is impossible for us to anticipate all factors that could affect our actual results. For a discussion of potential risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the “Risk Factors” section, as well as discussions of potential risks, uncertainties and other important factors, in our most recent filings with the Securities and Exchange Commission. All forward-looking statements included in this presentation represent our views as of the date hereof and should not be relied upon as representing our views as of any date subsequent to the date on the cover page of this presentation. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so.

No representation or warranty is made as to the accuracy or completeness of the information or analysis in this presentation.

Presentation Overview

- Introduction to Solid Biosciences
- Background on dystrophin
- Gene transfer for the treatment of Duchenne
- Solid's SGT-001 microdystrophin gene therapy program and the IGNITE DMD clinical trial
- SGT-001 manufacturing

Purpose-Built to Solve Duchenne Muscular Dystrophy (DMD)



360-Degree Approach

Address all facets of DMD

Differentiated Lead Gene Transfer Program

Data from second dose cohort later this year

Scalable Manufacturing Process

Meet clinical and commercial needs

Solid Is Addressing the Full Spectrum of Duchenne

CORRECTIVE THERAPIES



Gene therapy to address the genetic cause of DMD

DISEASE UNDERSTANDING



Biomarkers and endpoints to improve development

DISEASE-MODIFYING THERAPIES

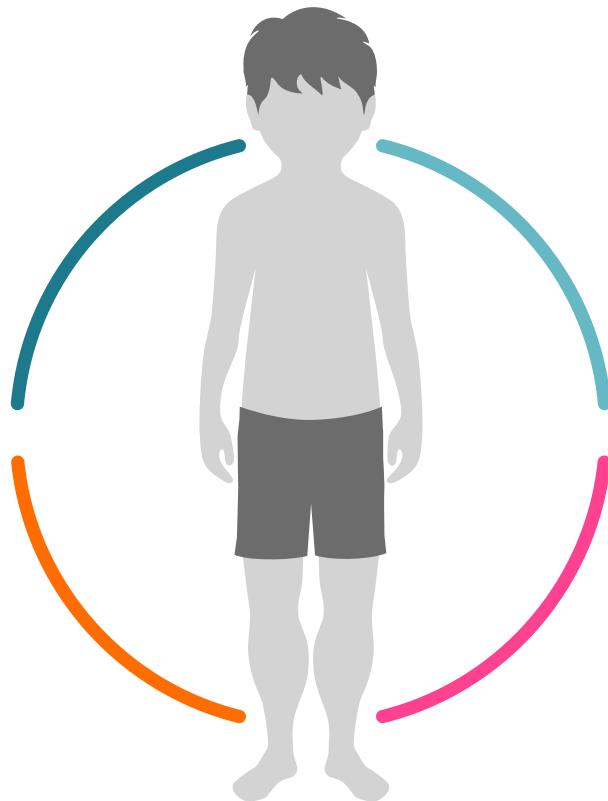


Small molecules and biologics to address disease mechanisms

ASSISTIVE DEVICES



Technology to support mobility



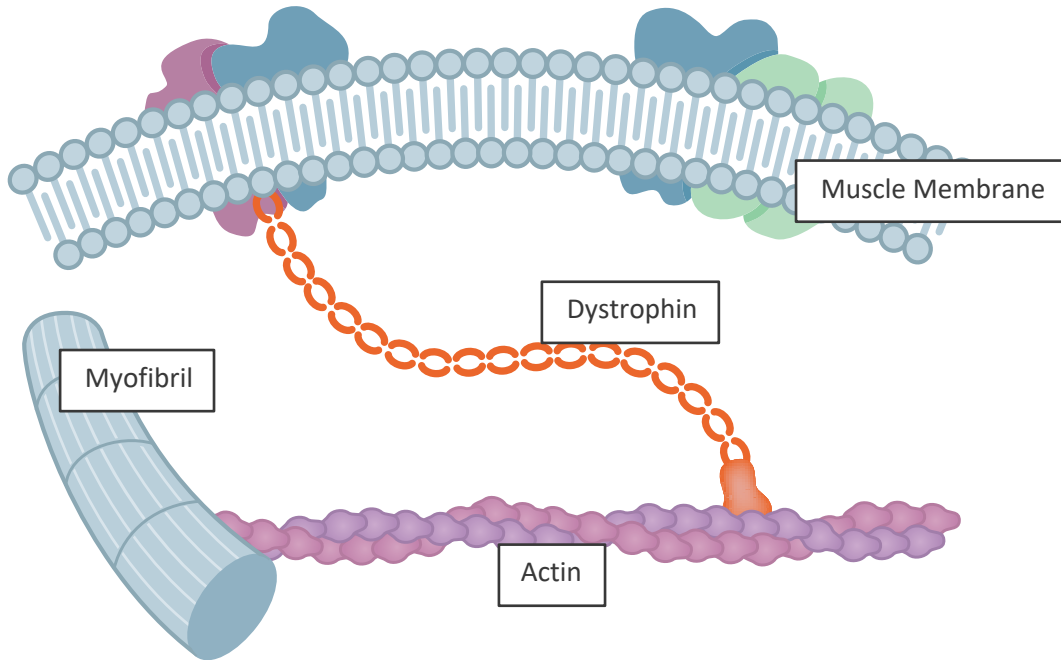
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The Importance of Dystrophin



Dystrophin Function in Healthy Muscle

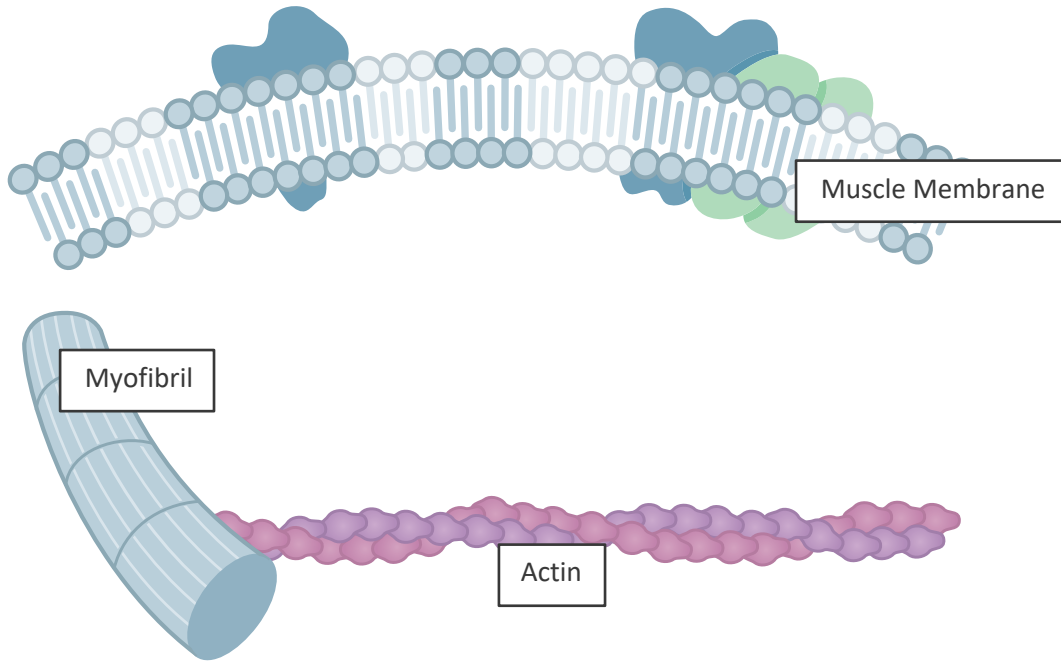
HEALTHY MUSCLE



- Dystrophin protects the muscle from damage and stabilizes critical dystrophin-associated proteins

Dystrophin is Missing in DMD Muscle

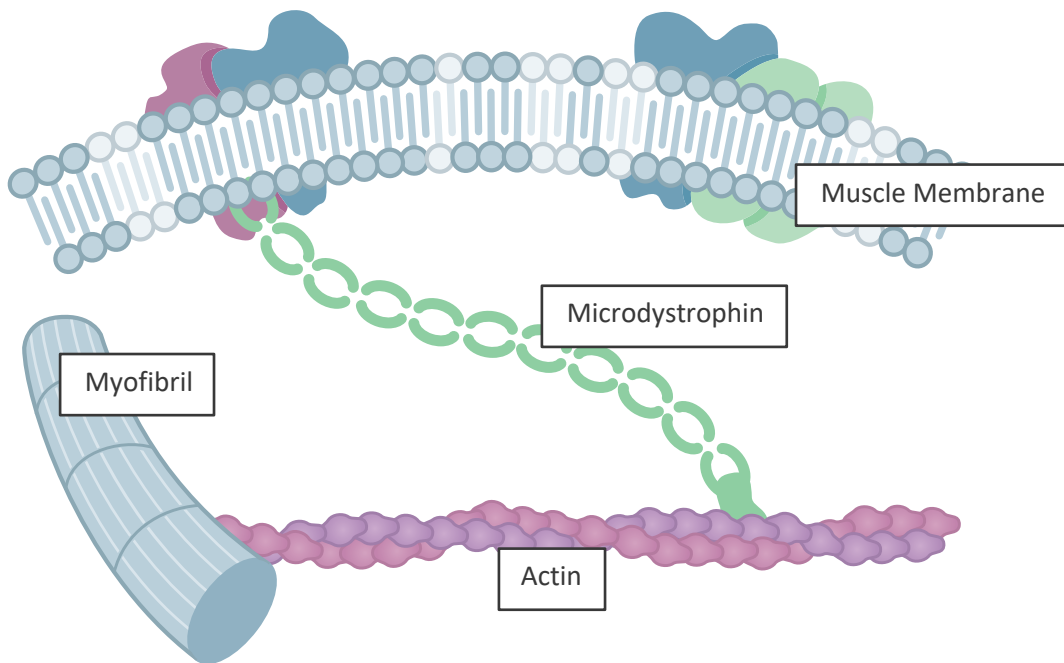
DYSTROPHIC MUSCLE



- In DMD, mutations in the dystrophin gene result in the absence of functional dystrophin protein
- Muscle fibers become damaged, cannot be repaired or replaced and are taken over by fat and scar tissue

Gene Transfer to Address the Genetic Cause of DMD

TREATED MUSCLE



- Gene transfer brings instructions to the cell to make a new kind of dystrophin designed to replace the missing dystrophin protein



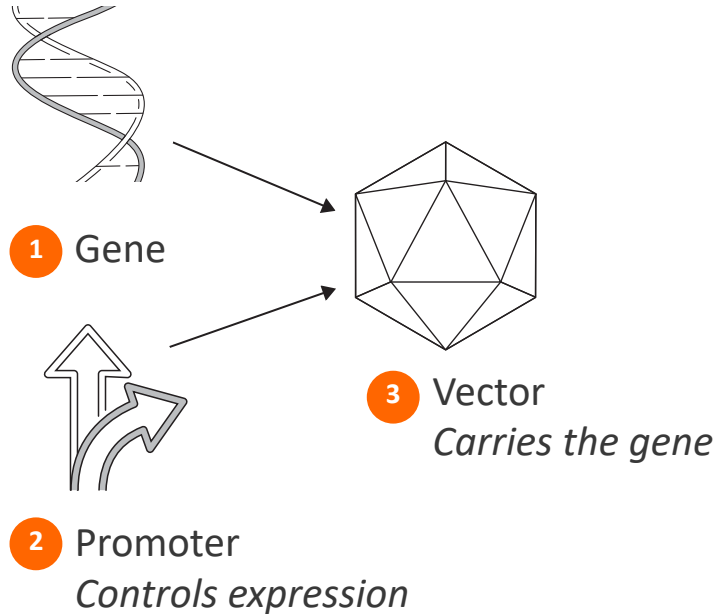
Corrective Therapies

Microdystrophin Gene Transfer

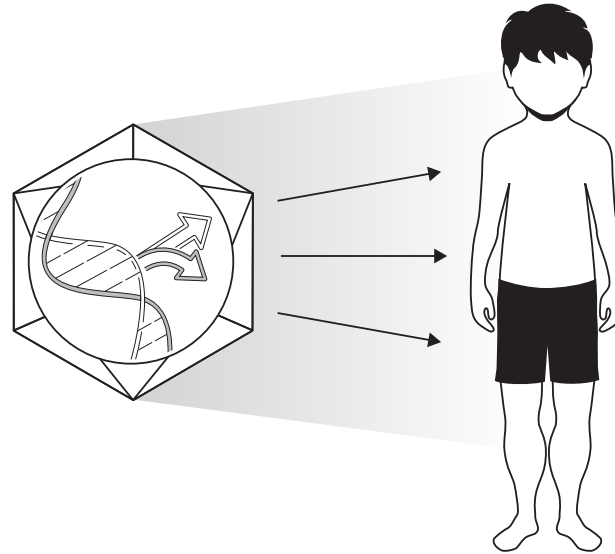


What Is Gene Transfer For DMD?

Gene transfer for DMD is made up of three essential elements:



The combined product is then given to the patient



Each Component of SGT-001 Was Carefully Selected



Transgene



Restore key functions
of a complex protein



SGT-001
microdystrophin gene



Promoter



Expression in skeletal
and heart muscle



CK8 promoter



Vector

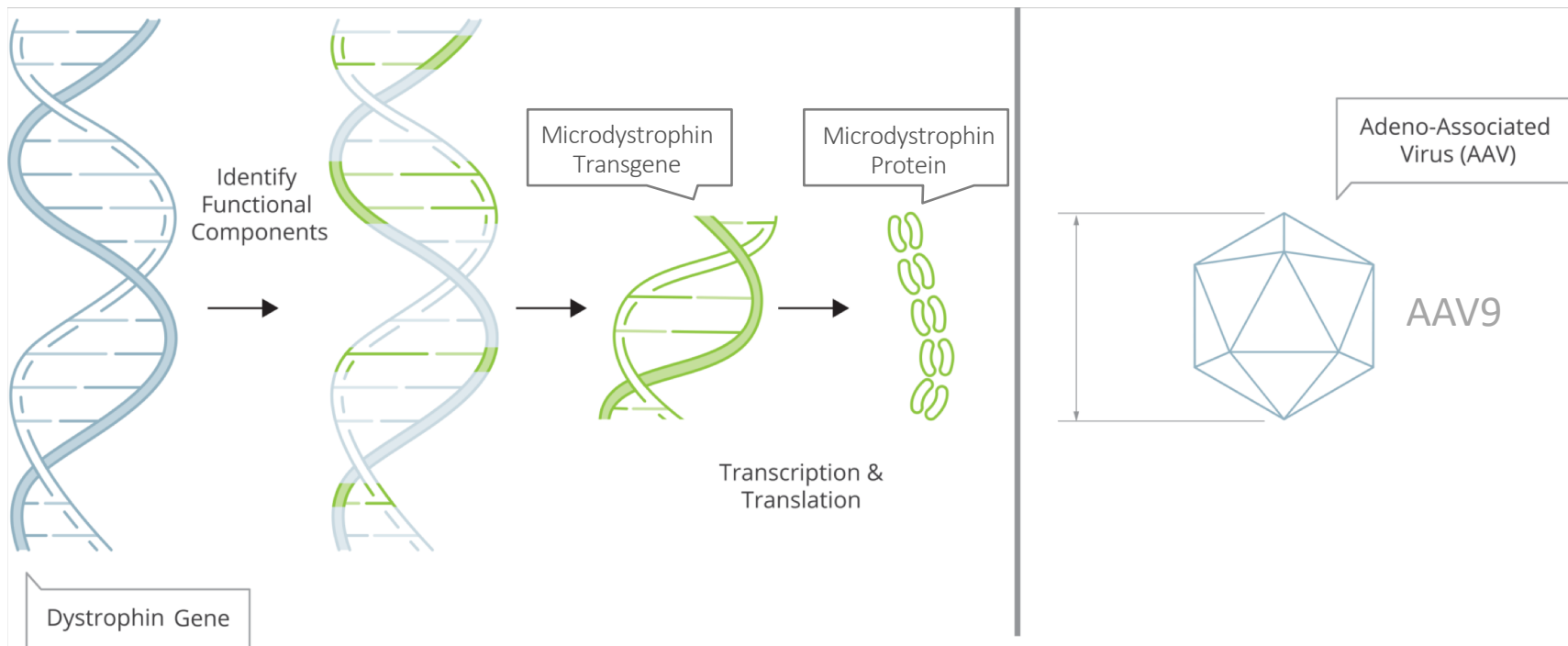


Targets skeletal and
heart muscle



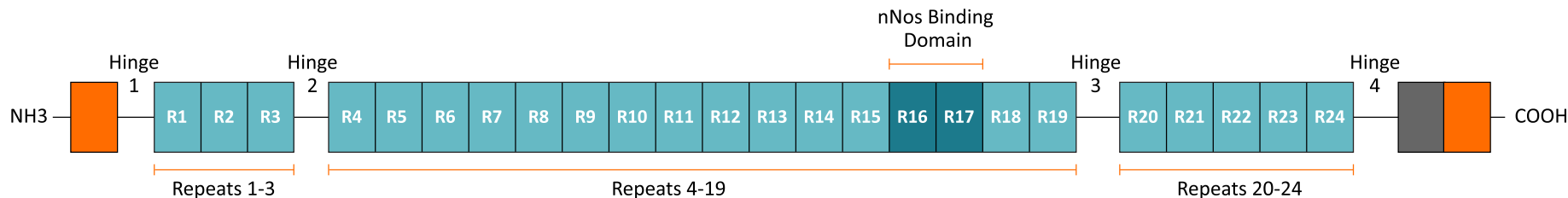
AAV9 vector

SGT-001 AAV-Mediated Microdystrophin Gene Therapy

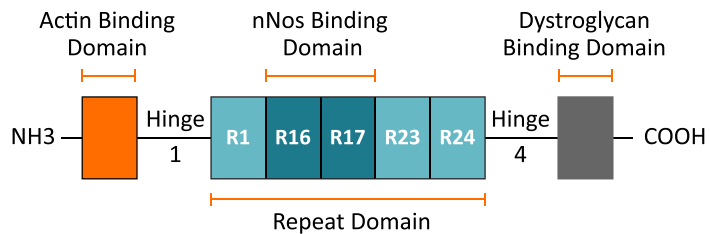


Features of SGT-001 Microdystrophin

Full Length Dystrophin Protein



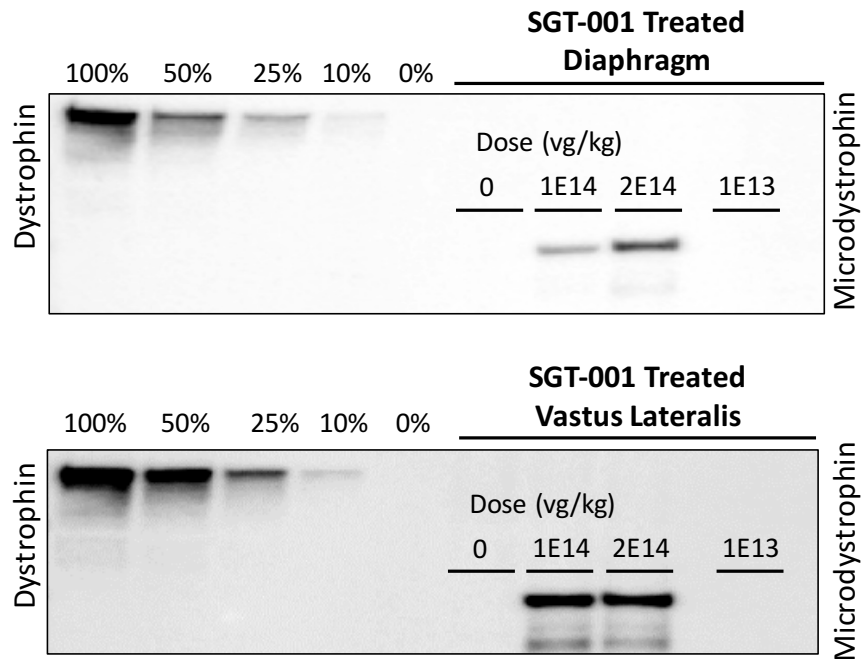
SGT-001 Microdystrophin Protein



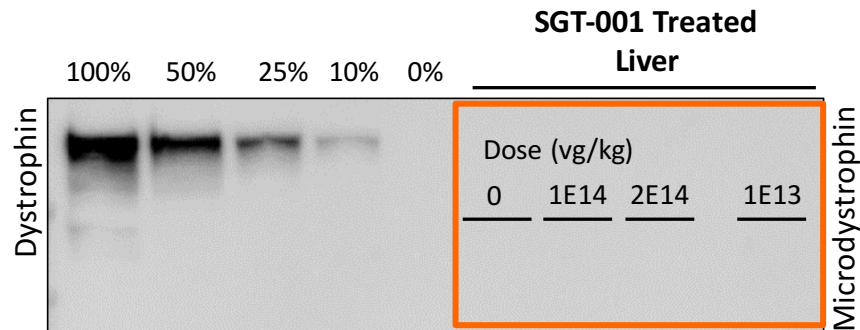
- SGT-001 selection based on more than 30 years of research; confirmed through comparison experiments by Solid

Animal Studies Show SGT-001 Microdystrophin is Made Selectively in Muscle

Target Tissue

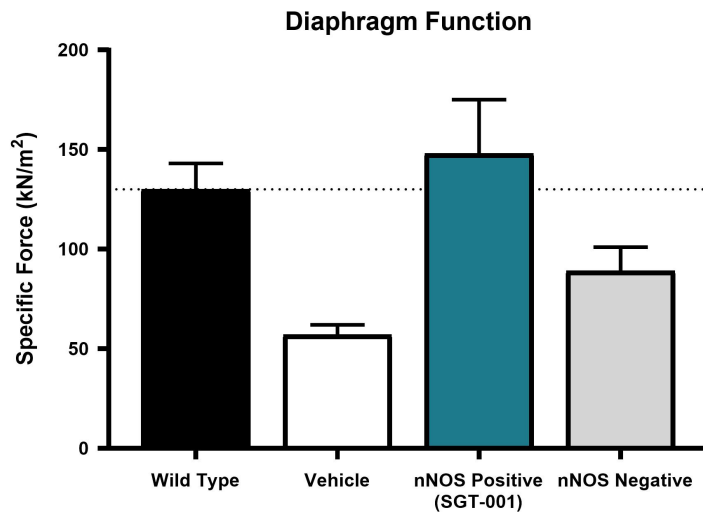


Non-target Tissue



SGT-001 Microdystrophin with nNOS Binding Domain Showed Greater Improvements in Muscle Strength in a Mouse Model of DMD

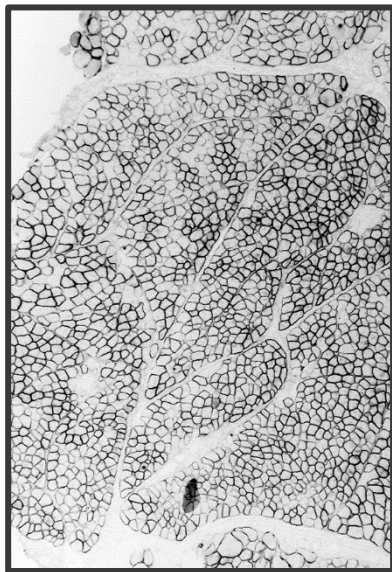
SGT-001 treatment led to force generation levels comparable to those in wild-type mice



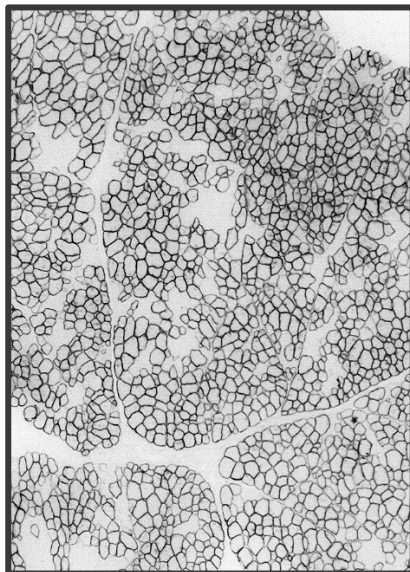
Specific diaphragm force 6 months post-treatment. Data shown as mean \pm SEM.
n=5-7 per group.

Microdystrophin Expression Lasts at Least 2.5 years in an Animal Model of DMD

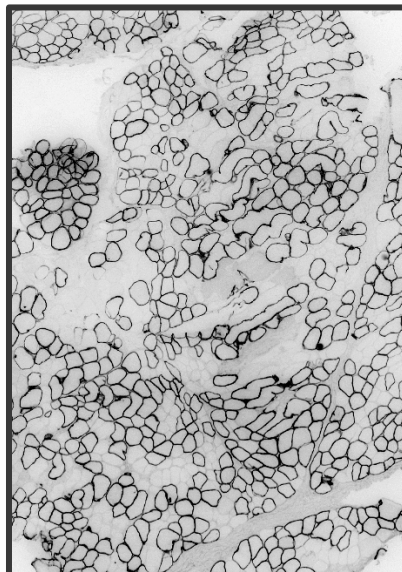
1 Month



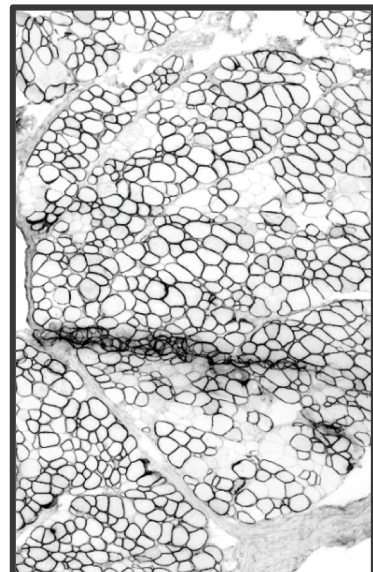
3 Months



24 Months



30 Months



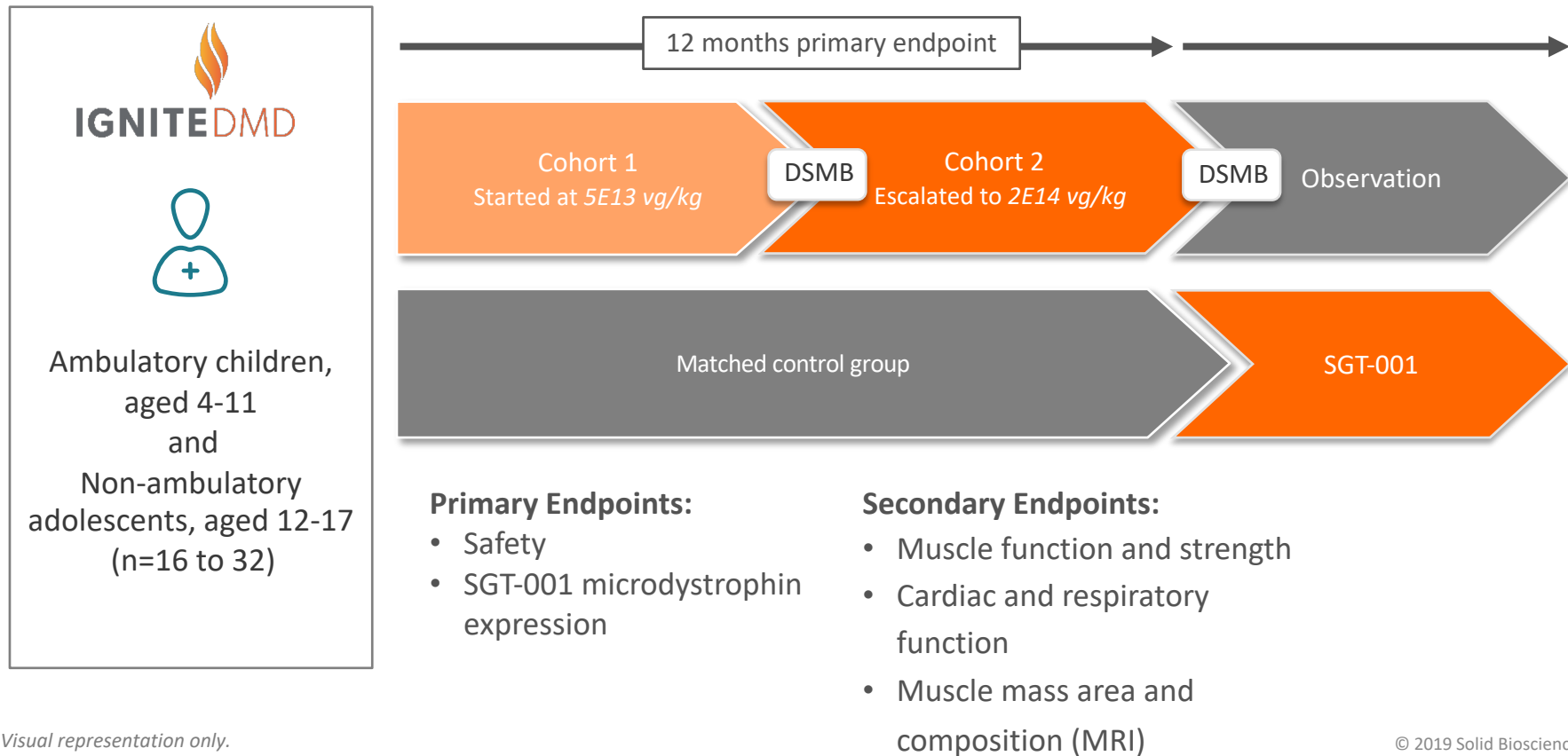


SGT-001 Clinical Program

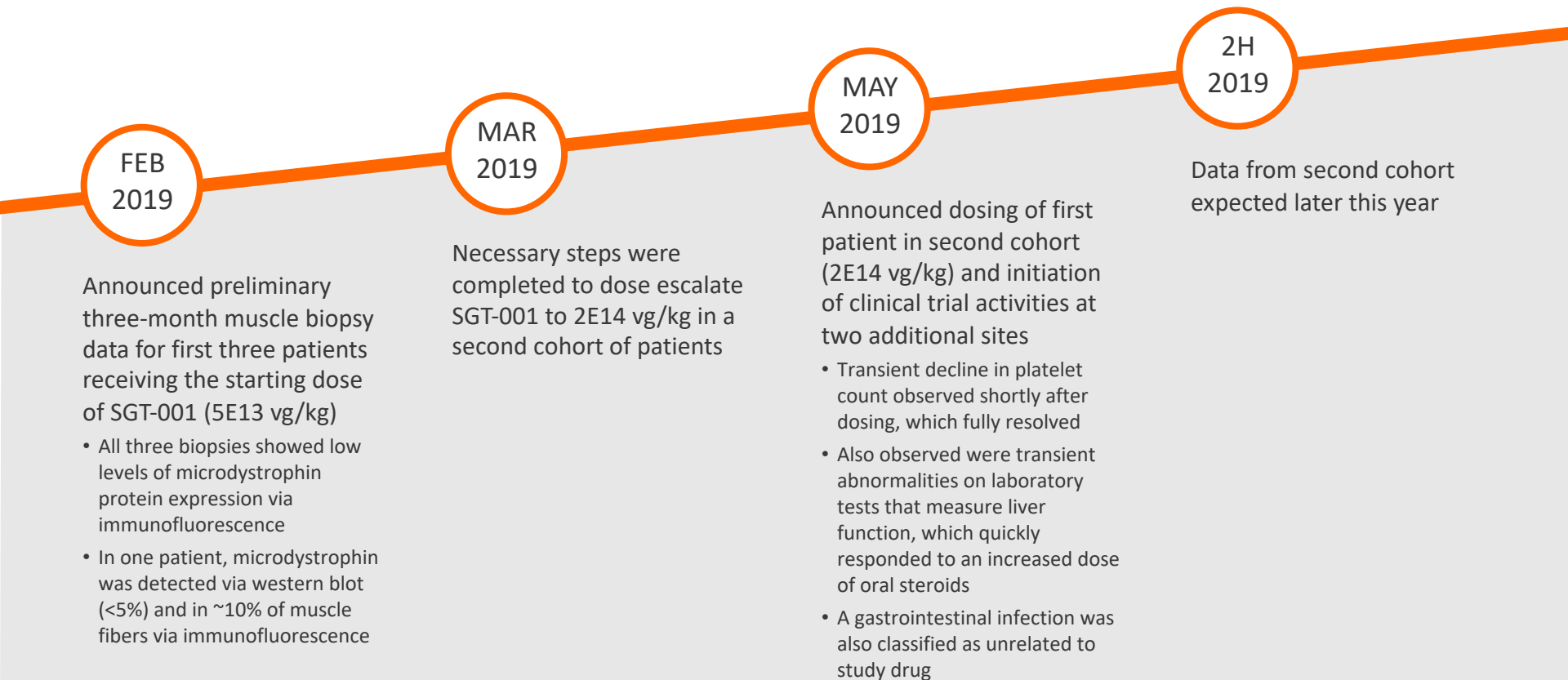
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IGNITE DMD



SGT-001 Phase I/II Clinical Study Ongoing



IGNITE DMD: Study Status



Manufacturing

Producing SGT-001

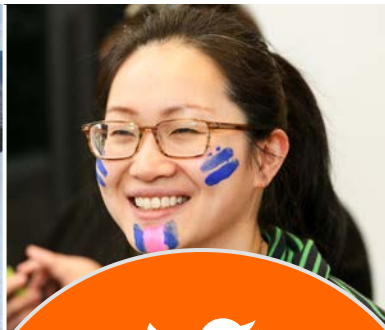


$$\left(\begin{array}{c} \text{HIGH} \\ \text{PATIENT NEED} \end{array} \right) \times \left(\begin{array}{c} \text{HIGH} \\ \text{AVERAGE PATIENT} \\ \text{WEIGHT} \end{array} \right) \times \left(\begin{array}{c} \text{HIGH} \\ \text{DOSES} \end{array} \right) = \begin{array}{c} \text{SIGNIFICANT} \\ \text{SUPPLY NEEDS} \end{array}$$

Solid Manufacturing Capability

- Successfully scaled up to 250L in suspension and produced multiple batches
- Each 250L batch can dose multiple patients
- Create ability to potentially treat 1,000s of patients

Thank you!



@SolidBioDMD
#TogetherWeAreSolid
#PatientPowered

