SOLID BIOSCIENCES

Solid Biosciences

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Parent Project Muscular Dystrophy

2019 ANNUAL CONFERENCE

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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

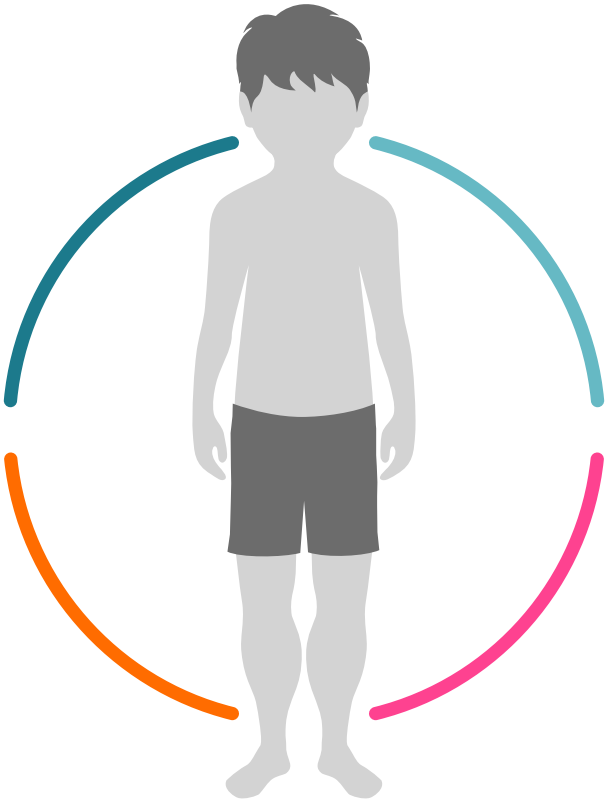
Solid Is Addressing the Full Spectrum of Duchenne

CORRECTIVE THERAPIES

Gene therapy to address the genetic cause of DMD

DISEASE-MODIFYING THERAPIES

Small molecules and biologics to address disease mechanisms



DISEASE UNDERSTANDING

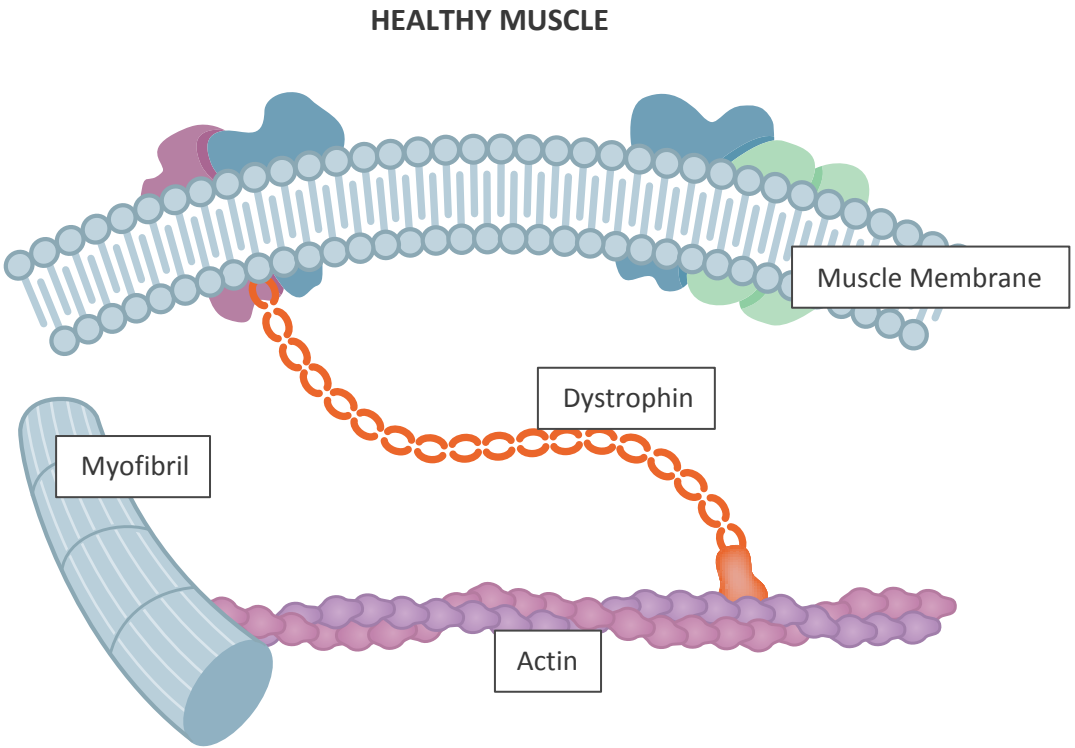
Biomarkers and endpoints to improve development

ASSISTIVE DEVICES

Technology to support mobility

The Importance of Dystrophin

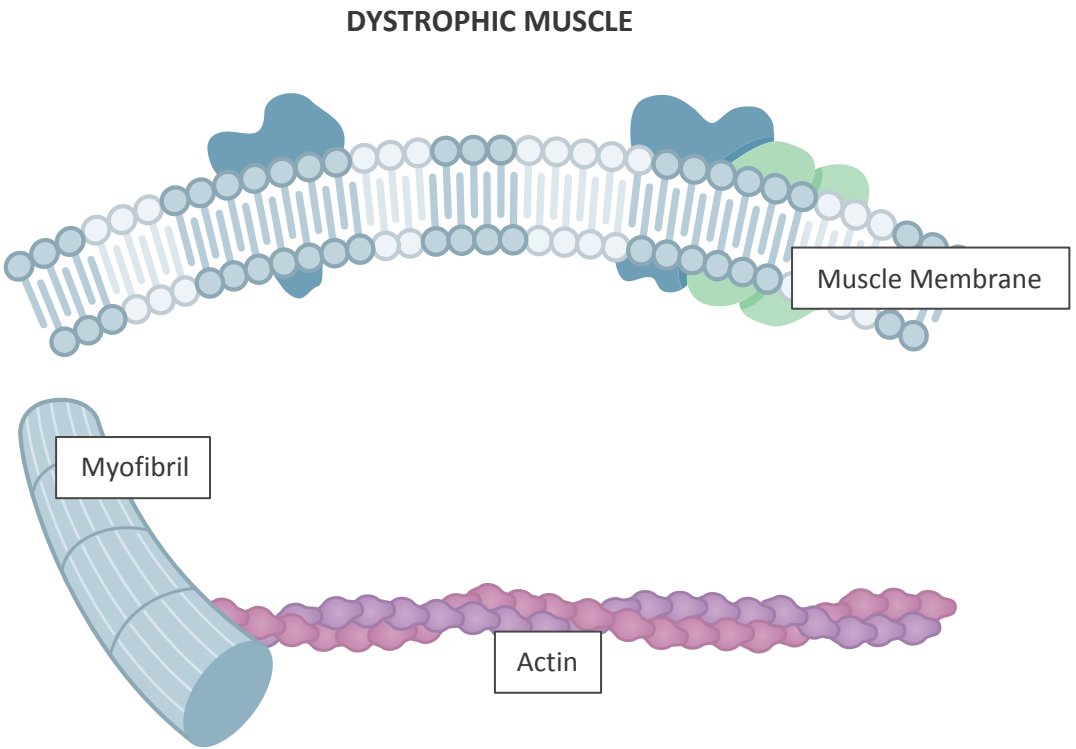
Dystrophin Function in Healthy Muscle



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

Visual representation only

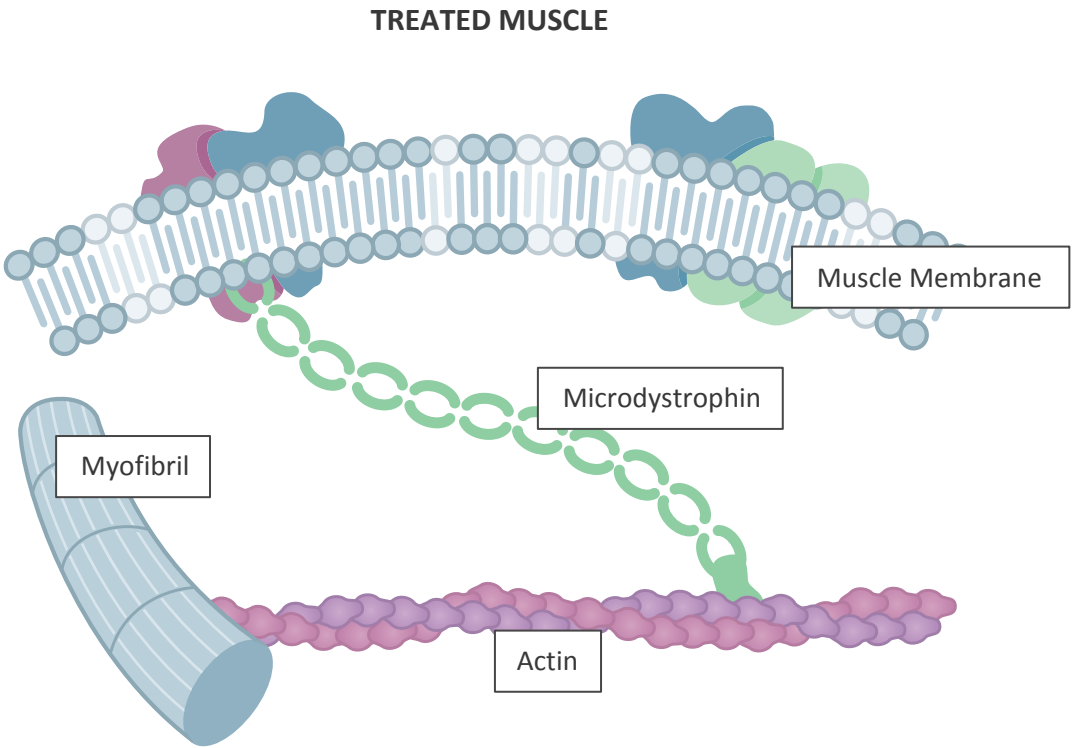
Dystrophin is Missing in DMD Muscle



- 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

Visual representation only

Gene Transfer to Address the Genetic Cause of DMD



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

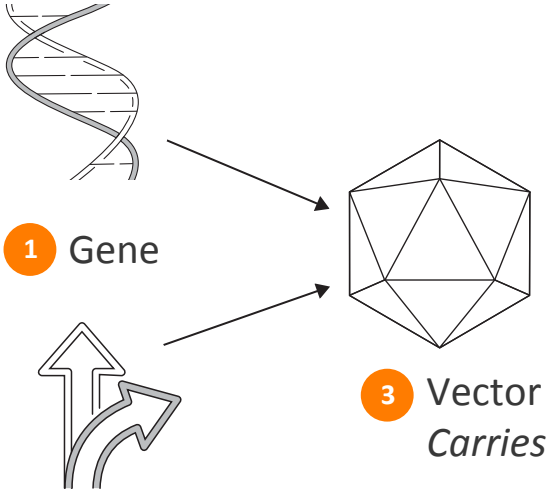
Visual representation only

Corrective Therapies

Microdystrophin Gene Transfer

What Is Gene Transfer For DMD?

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

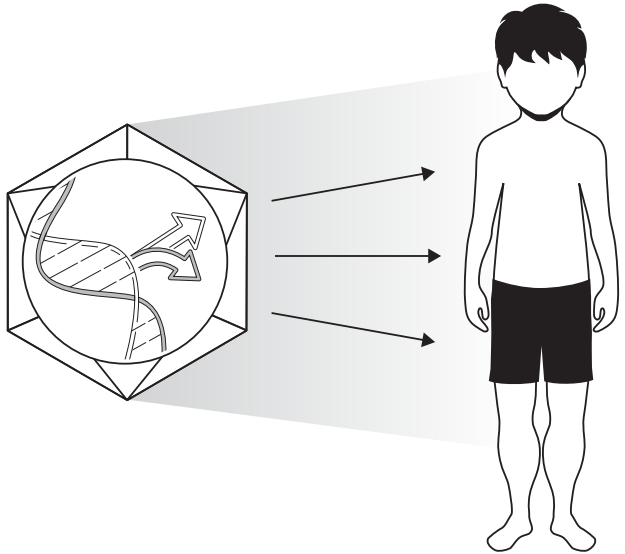


1 Gene

2 Promoter
Controls expression

3 Vector
Carries the gene

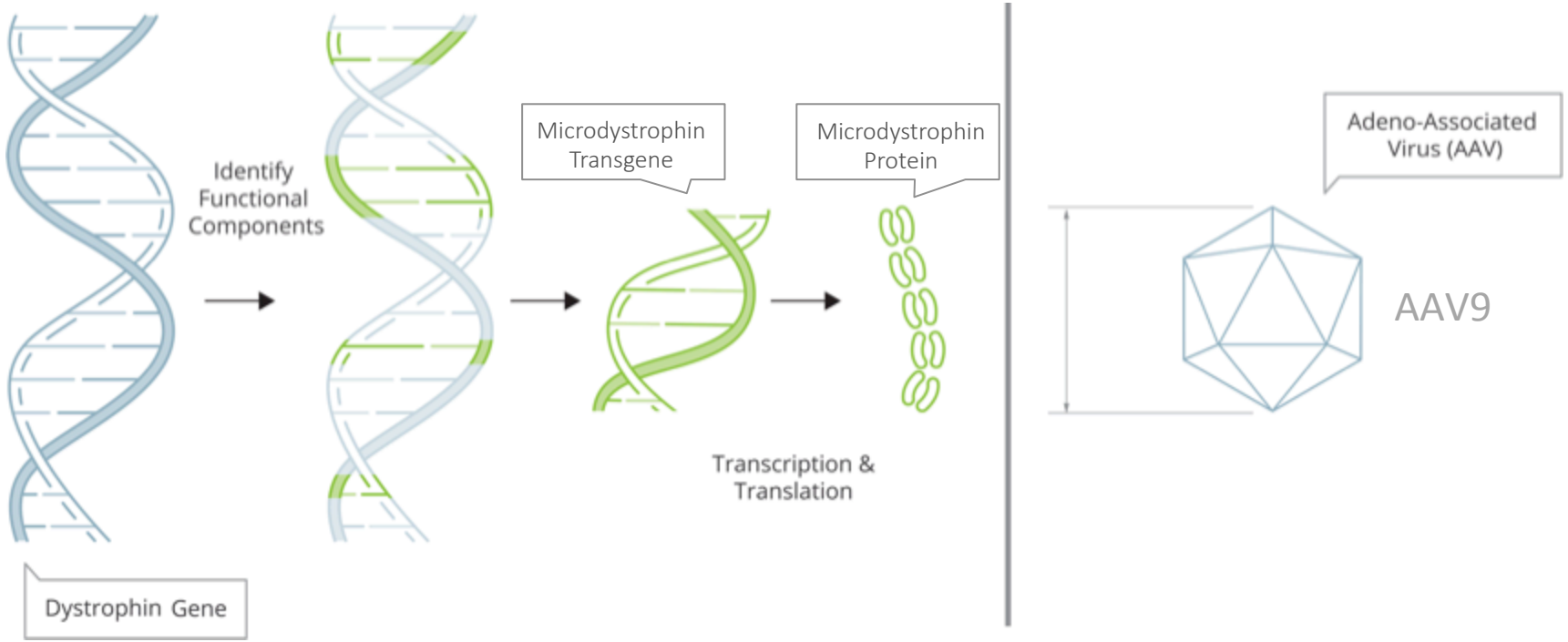
The combined product is then given to the patient



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



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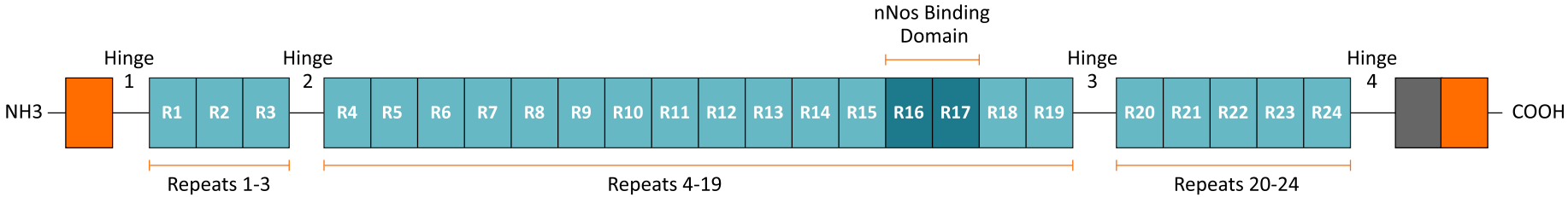
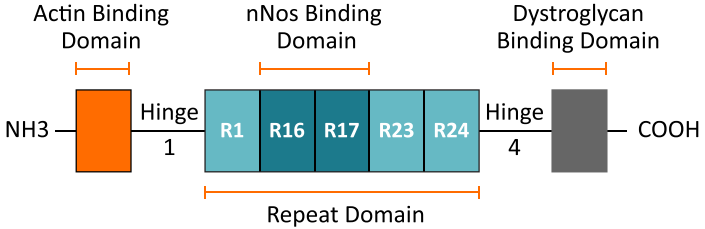
Features of SGT-001 Microdystrophin

Full Length Dystrophin Protein



SGT-001 Microdystrophin Protein

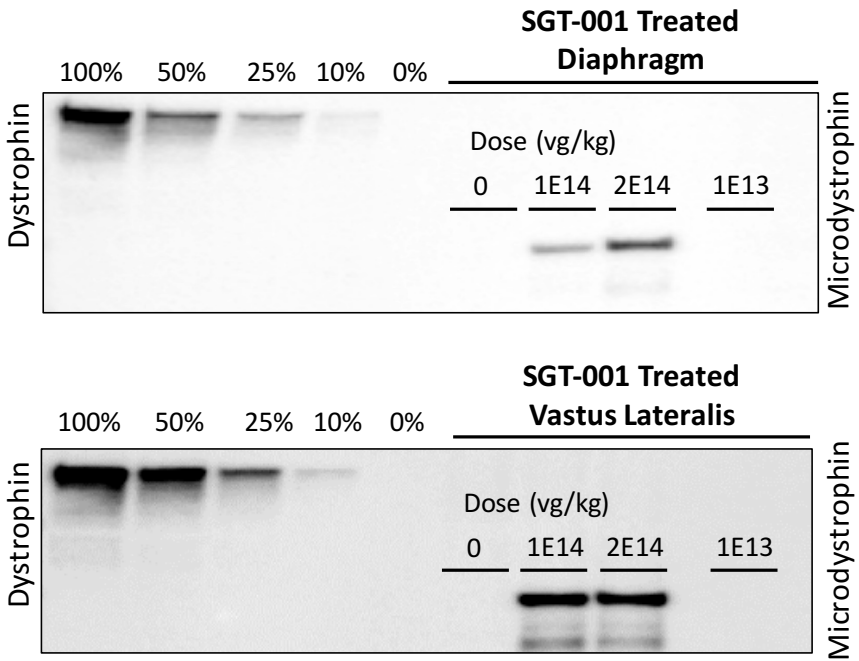


- SGT-001 selection based on more than 30 years of research; confirmed through comparison experiments by Solid

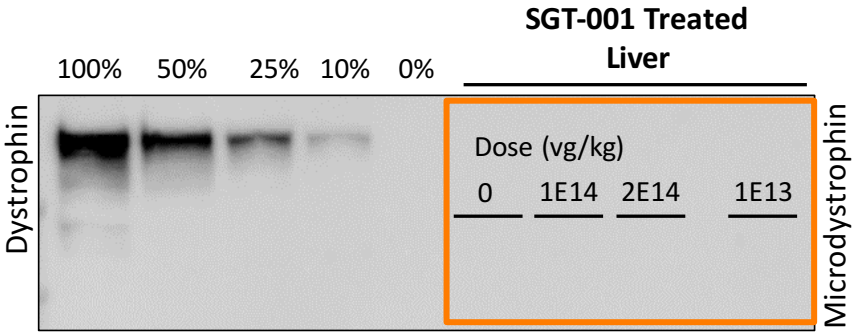
Visual representation only.

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

Target Tissue



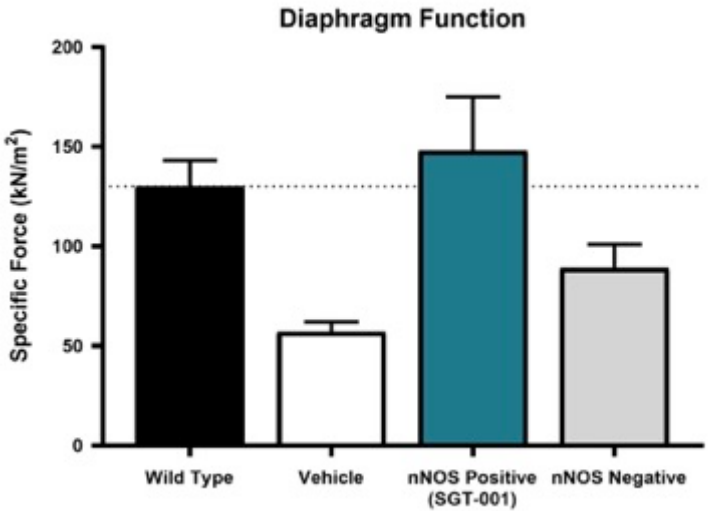
Non-target Tissue



Company data. Three month efficacy study in GRMD canines. Representative only.

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

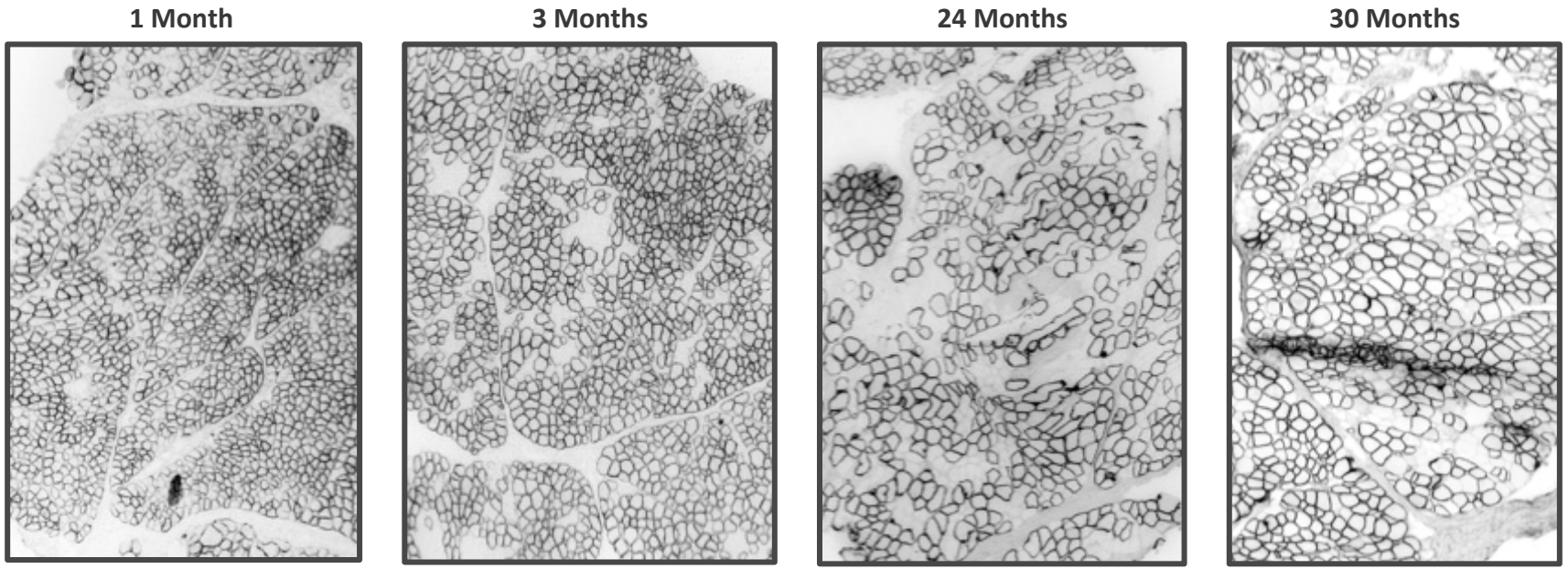
SGT-001 treament led to force generation levels comparable to those in wild-type mice



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

Ramos et al. Molecular Therapy, 2019.

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



Hakim et al. American Society of Gene and Cell Therapy (ASGCT), 2018.

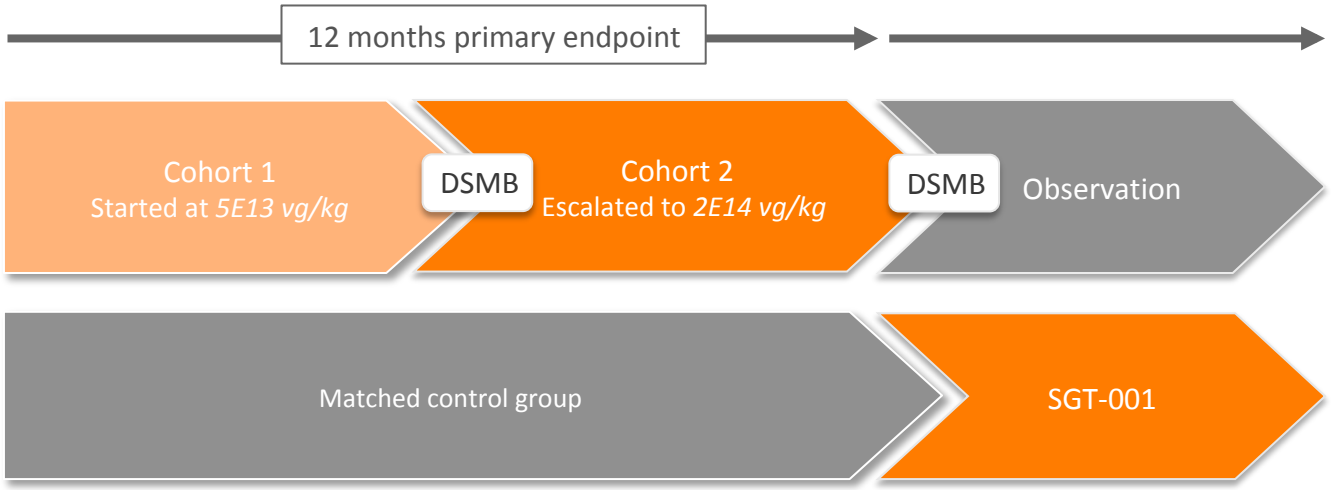
SGT-001 Clinical Program

IGNITE DMD

SGT-001 Phase I/II Clinical Study Ongoing

IGNITEDMD

Ambulatory children, aged 4-11
and
Non-ambulatory adolescents, aged 12-17
(n=16 to 32)



Primary Endpoints:

- Safety
- SGT-001 microdystrophin expression

Secondary Endpoints:

- Muscle function and strength
- Cardiac and respiratory function
- Muscle mass area and composition (MRI)

Visual representation only.

IGNITE DMD: Study Status

FEB 2019

Announced preliminary three-month muscle biopsy data for first three patients receiving the starting dose of SGT-001 (5E13 vg/kg)

- All three biopsies showed low levels of microdystrophin protein expression via immunofluorescence
- In one patient, microdystrophin was detected via western blot (<5%) and in ~10% of muscle fibers via immunofluorescence

MAR 2019

Necessary steps were completed to dose escalate SGT-001 to 2E14 vg/kg in a second cohort of patients

MAY 2019

Announced dosing of first patient in second cohort (2E14 vg/kg) and initiation of clinical trial activities at two additional sites

- Transient decline in platelet count observed shortly after dosing, which fully resolved
- Also observed were transient abnormalities on laboratory tests that measure liver function, which quickly responded to an increased dose of oral steroids
- A gastrointestinal infection was also classified as unrelated to study drug

2H 2019

Data from second cohort expected later this year

Manufacturing

Producing SGT-001

Addressing the DMD Gene Therapy Supply Challenge

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
#PatientPowereed