July
2018

Solid Biosciences

Corporate Overview

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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, 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.

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Solid Biosciences: A Singular Commitment to Cure Duchenne Muscular Dystrophy

360-Degree Approach to DMD

- Targeting underlying genetic cause and all disease manifestations

DMD Center of Excellence

- Experienced management team
- World-renowned scientific/clinical/community advisors

Advancing & Expanding Pipeline

- Rigorous selection process
- SGT-001 Ph I/II clinical trial (IGNITE DMD) initiated in Nov 2017
  - Microdystrophin gene transfer, mutation agnostic
  - Data driven: expression correlated with functional improvement in multiple large and small animal models
- SB-001 antibody against fibrosis; preclinical development to begin in 2018
- Progressing pipeline of early-stage next generation gene therapies and complementary programs

Scalable GMP Manufacturing Process

- Preparing to meet potential commercial needs

Resourced to Fund Growth

- ~$129.1mm in net proceeds from IPO in Jan 2018

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Committed To Patients With Duchenne Muscular Dystrophy

About Duchenne Muscular Dystrophy (DMD)

1 in 3,500-5,000 boys born

10,000-15,000 cases in the U.S.

No geographic or ethnic boundaries

Inherited or spontaneous X-linked mutation

More than a thousand different mutations in the dystrophin gene can lead to DMD

Progressive & irreversible

Diagnosis at 3-5 years old

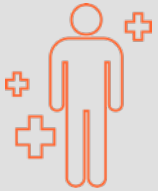
Wheelchair in teens; ventilator in 20's

100% fatal

Economic burden*

More than $1B a year in the United States

Impacts patients, families, caregivers and the healthcare system



Significant Unmet Need

Glucocorticoids are standard of care

Approved disease modifying therapies target only small subsets of patients

Source: Erik Landfeldt, Peter Lindgren, Christopher F. Bell, et al. The burden of Duchenne muscular dystrophy: An international, cross-sectional study. Neurology 2014.

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Solid Is A Center Of Excellence In DMD

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Charity Partners

Parent Project Muscular Dystrophy

Joiningjack.

Alex's Wish
His wish, our mission

DUCHENNE UK

Duchenne Research Fund

Duchenne Now
because tomorrow's too late

Join Our Boys

SAVE OUR SONS
DUCHENNE AUSTRALIA

FIGHT DMD

Research Collaborators*

SickKids

Kennedy Krieger Institute

Mizzou
University of Missouri

TEXAS A&M UNIVERSITY

University of Massachusetts Medical School
UMASS

UNIVERSITY of WASHINGTON

Synpromics
the leader in gene control

UF UNIVERSITY of FLORIDA

ADIMAB

sesmc
powered clothing™

CDMD

MEDICAL COLLEGE OF WISCONSIN

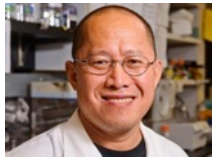
Massachusetts Eye and Ear

Lonza

*Sample Representation

Scientific Advisors

University of Washington
Jeff Chamberlain, Ph.D.



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Mass General Hospital
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Medical College of Wisconsin
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Manufacturing Consultant
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Solid's 360° Approach To Addressing DMD

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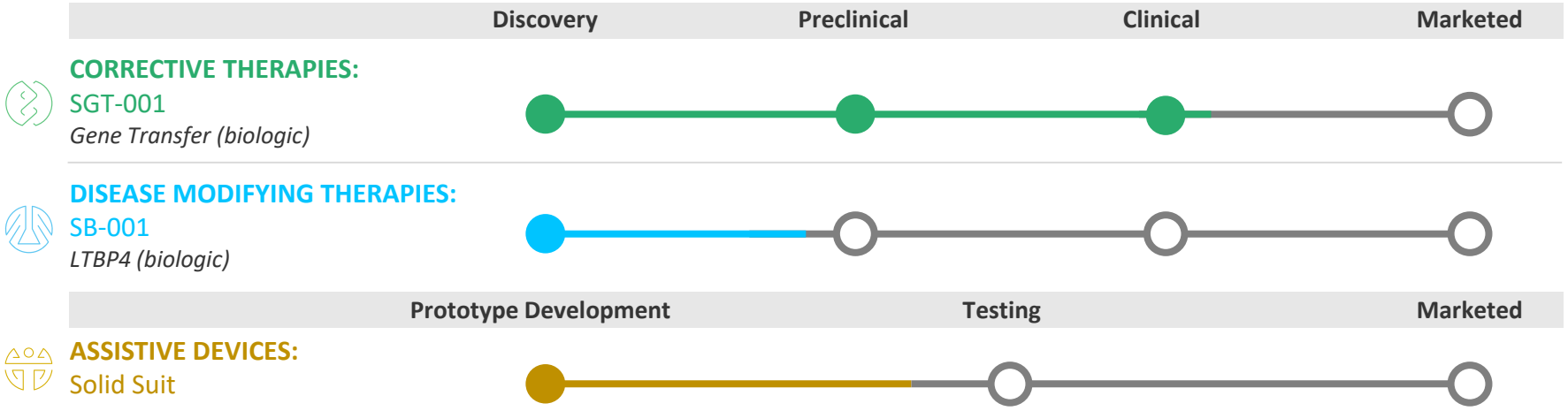
Solid Pipeline And Exploratory Programs

Pipeline



Exploratory Pre-development Programs

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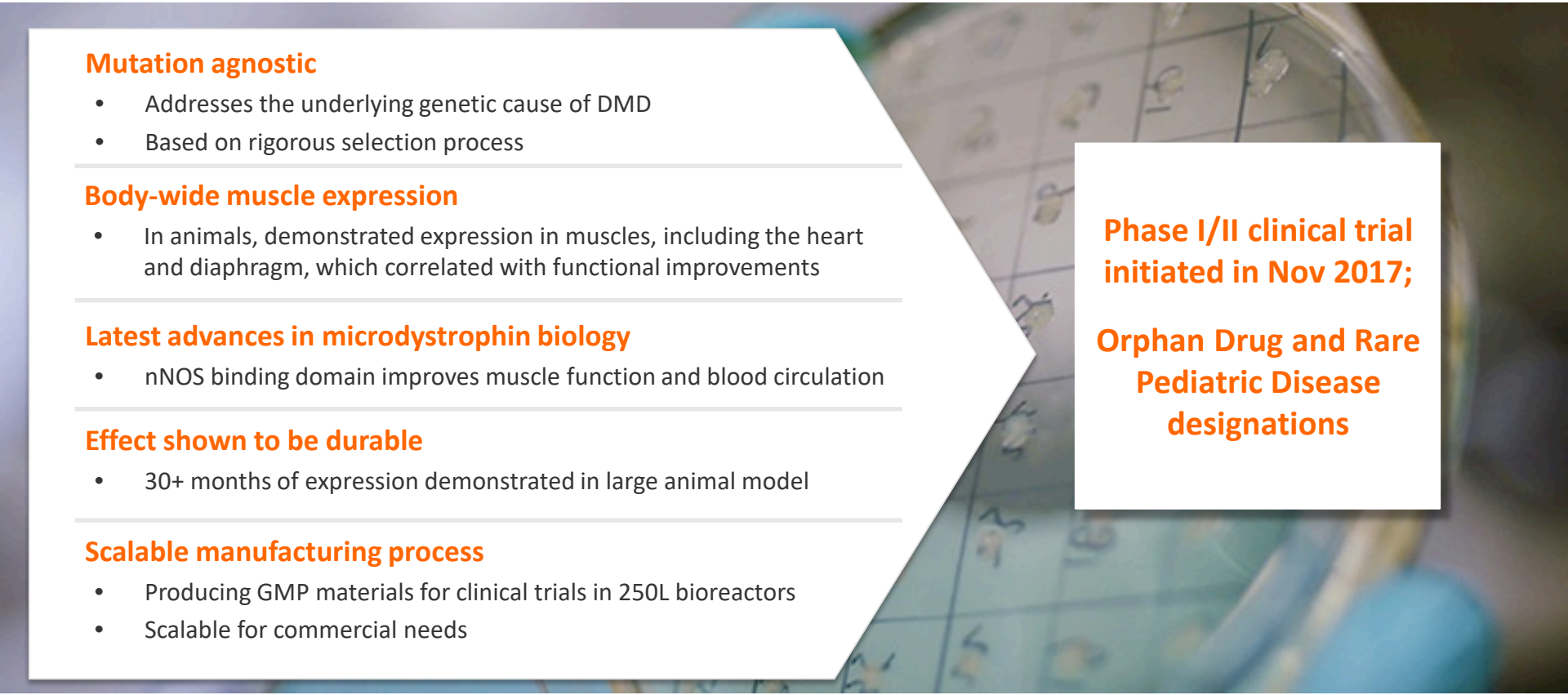
CORRECTIVE THERAPIES

Innovation In Gene Transfer

SGT-001 (Microdystrophin Gene Transfer)



Mutation agnostic

- Addresses the underlying genetic cause of DMD
- Based on rigorous selection process

Body-wide muscle expression

- In animals, demonstrated expression in muscles, including the heart and diaphragm, which correlated with functional improvements

Latest advances in microdystrophin biology

- nNOS binding domain improves muscle function and blood circulation

Effect shown to be durable

- 30+ months of expression demonstrated in large animal model

Scalable manufacturing process

- Producing GMP materials for clinical trials in 250L bioreactors
- Scalable for commercial needs

Phase I/II clinical trial initiated in Nov 2017;

Orphan Drug and Rare Pediatric Disease designations

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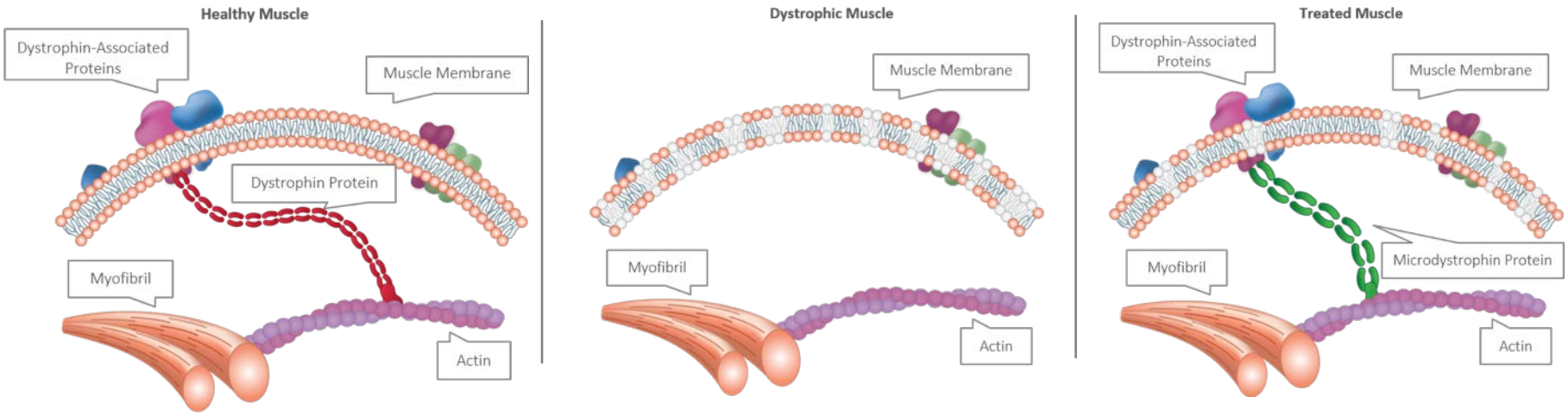
SGT-001 Is Designed To Address The Genetic Cause Of DMD



- In DMD, mutations in the dystrophin gene result in the loss of functioning dystrophin protein
- Without dystrophin, muscle fibers become unstable, lose the ability to repair themselves and ultimately die
- SGT-001 delivers a synthetic microdystrophin transgene that encodes for a functional dystrophin protein surrogate designed to replace dystrophin

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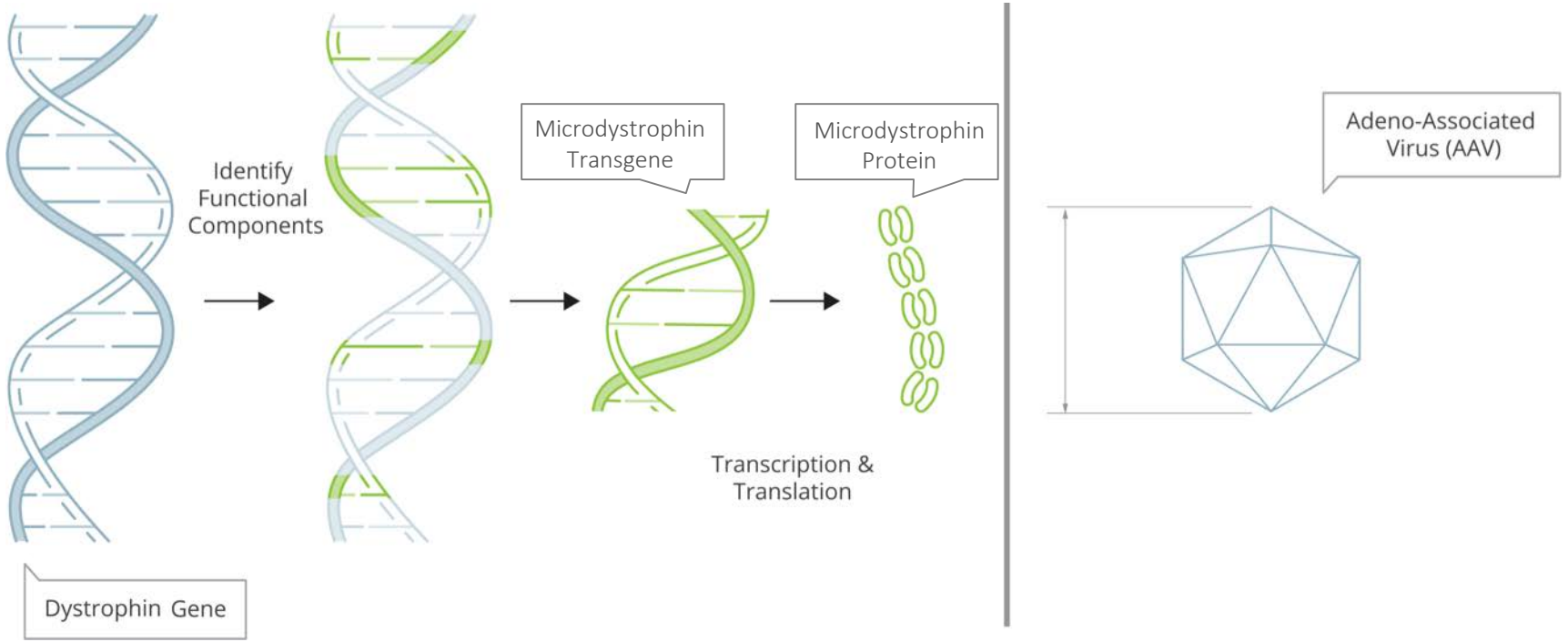
Microdystrophin Is Rationally Designed



The natural dystrophin gene is too large to fit into AAV, a common delivery mechanism for systemic gene therapy. Microdystrophin is rationally designed to fit into rAAV9 packaging constraints.

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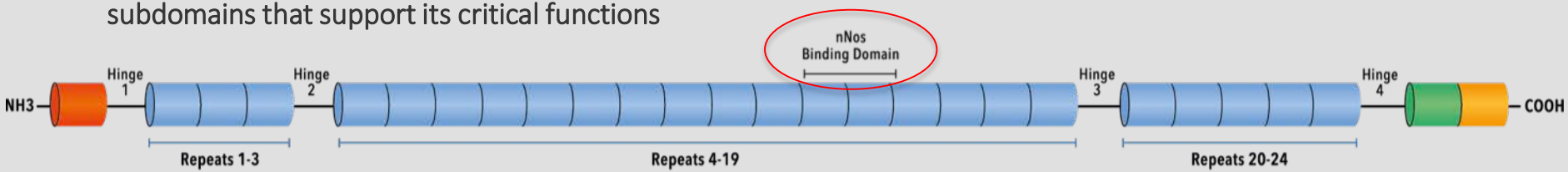
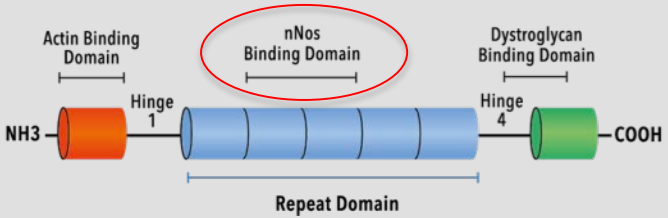
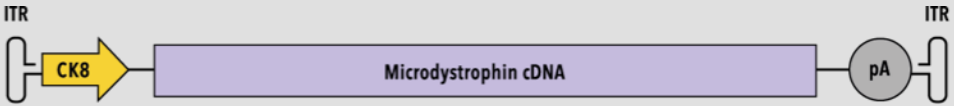
SGT-001 Composition

Dystrophin is a large protein comprised of many subdomains that support its critical functions



The SGT-001 transgene retains key components of dystrophin, including the elements necessary to promote nNOS activity. In healthy muscle expressing dystrophin, nNOS promotes muscle function and blood circulation





SGT-001 is regulated by a muscle-specific promoter, CK8, which restricts expression to skeletal and cardiac muscle

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Comprehensive IND-Enabling Preclinical Program

Wild-type and Dystrophic Mice	• Expression • Dose Response • Function • Kinetics • GLP Toxicology • Manufacturing Comparability
Mixed Breed Dystrophic Canine (cDMD) Golden Retriever Muscular Dystrophy (GRMD)	• Expression • Dose Response • Function • Durability
Non-human Primates	• GLP Toxicology

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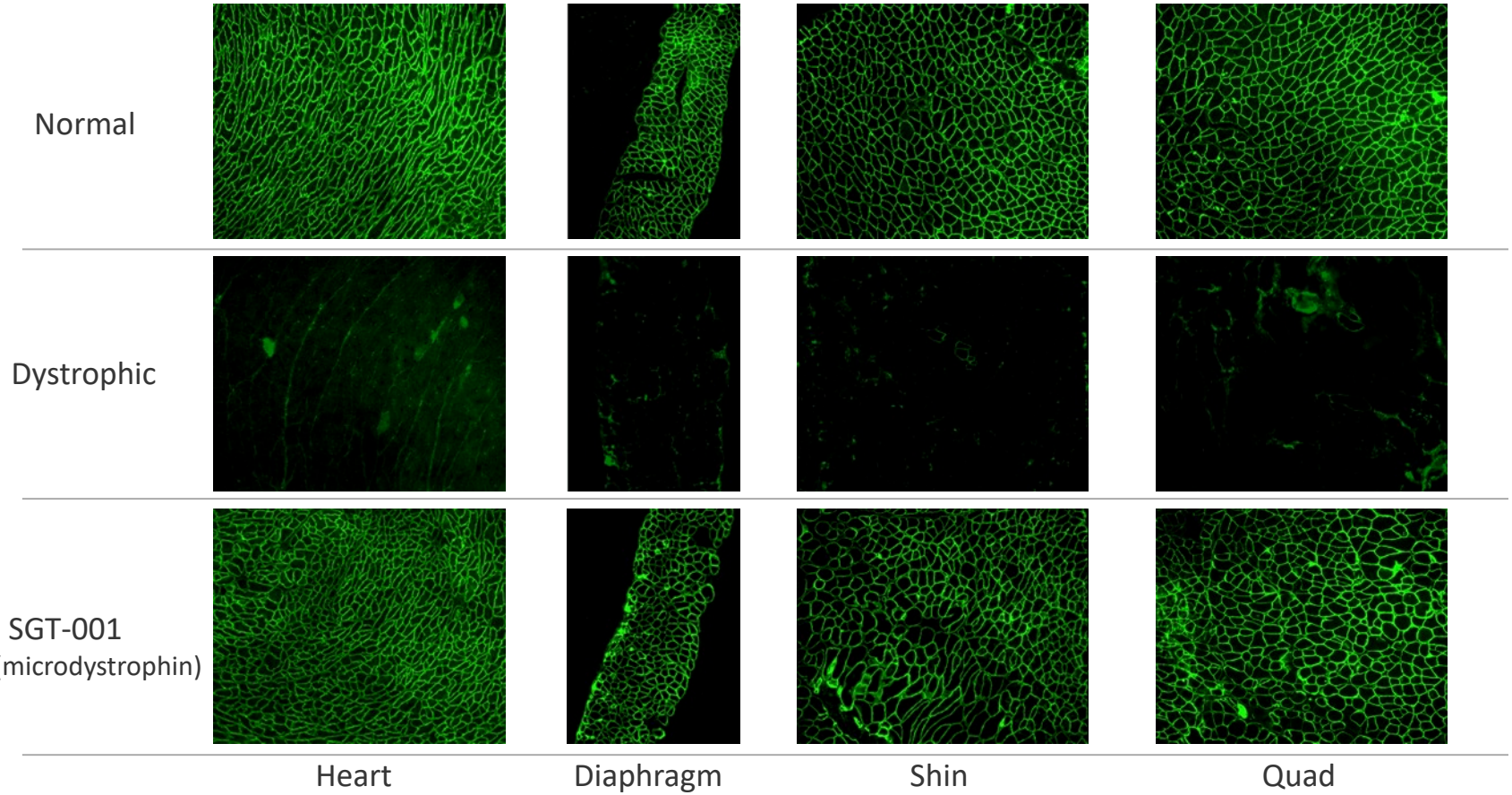
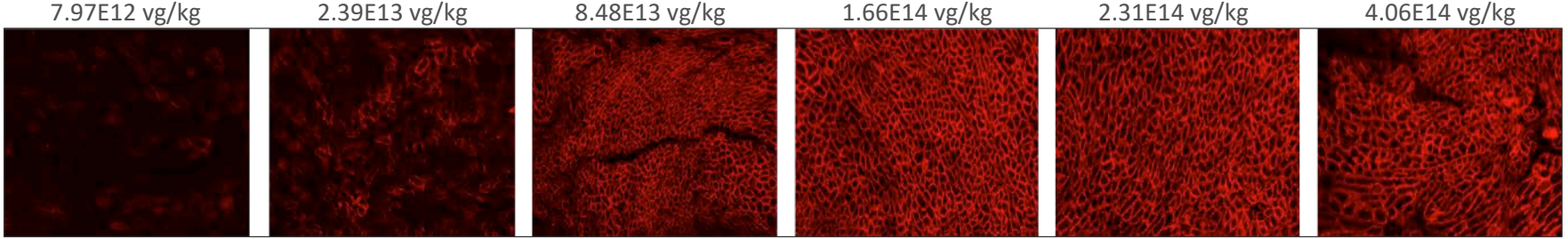
Demonstrated Wild-type Levels Of SGT-001 Expression In Mice



Source: A Five-Repeat Micro-Dystrophin Gene Ameliorated Dystrophic Phenotype in the Severe DBA/2J-mdx Model of Duchenne Muscular Dystrophy. Journal of Molecular Therapy Methods & Clinical Development

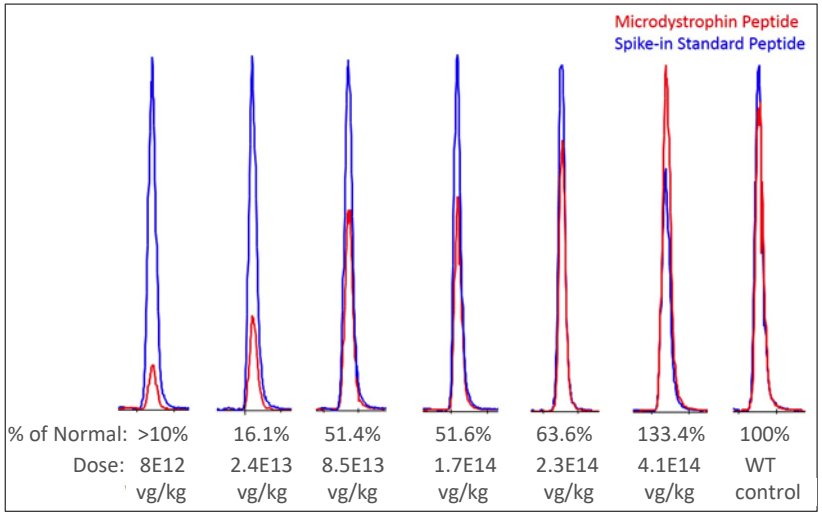
SGT-001 Expression Quantifiable Across Three Different Assays

Immunofluorescence



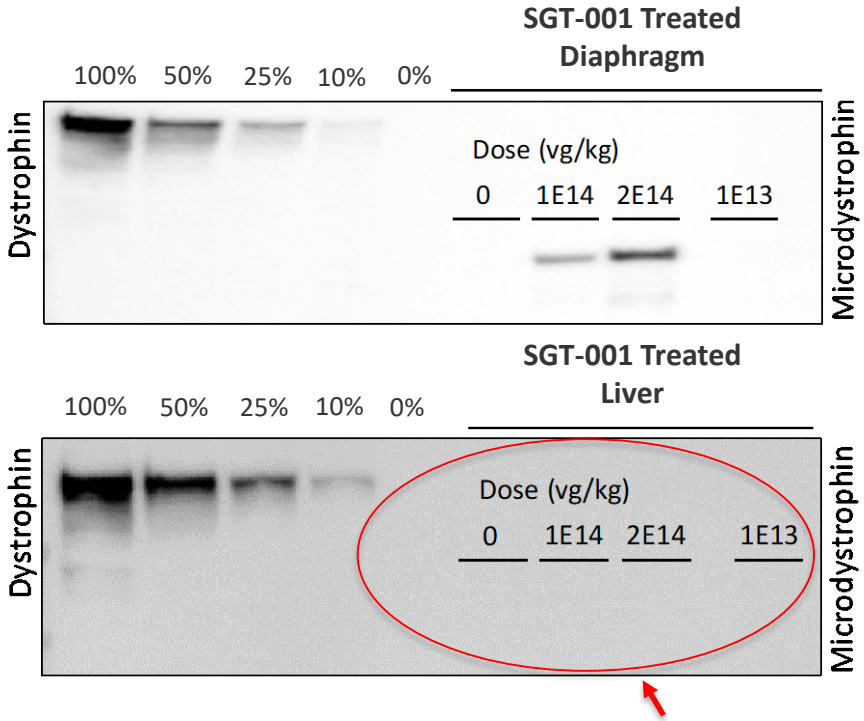
Western Blot

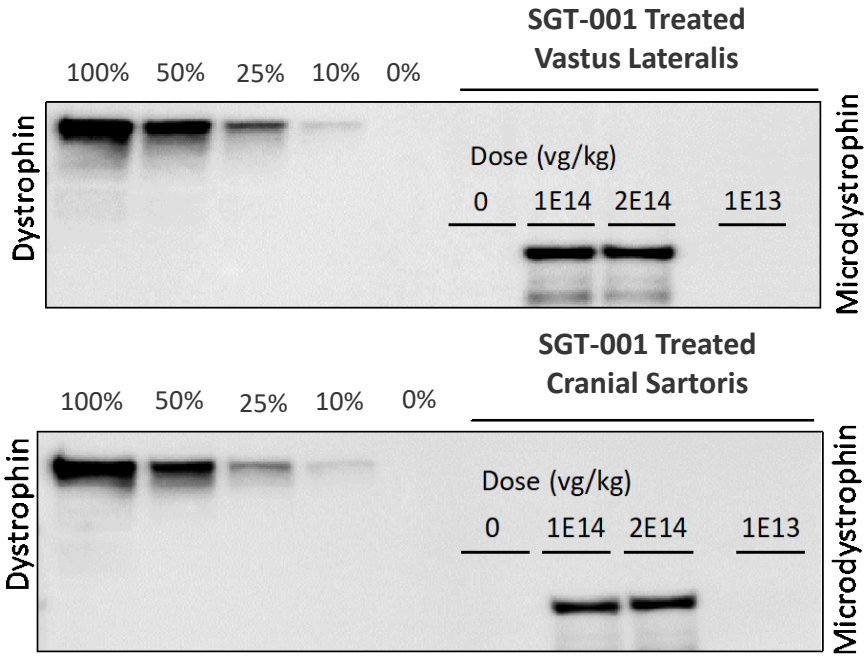
Mass Spectrometry



*Samples analyzed 28 days post administration; all the samples from same study

CK8 Muscle Specific Promoter Restricted Expression To Muscles In Dystrophic Canines





Preclinical studies have shown SGT-001 is expressed in skeletal and cardiac muscle, but is not expressed in non-skeletal muscle like the liver.

**Samples analyzed 90 days post administration*

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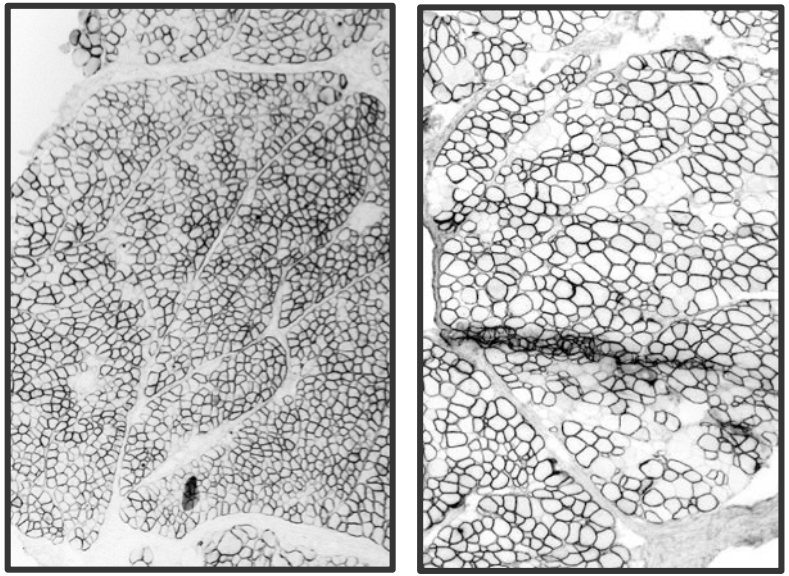
Sustained Expression Correlated With Functional Benefit In Canines

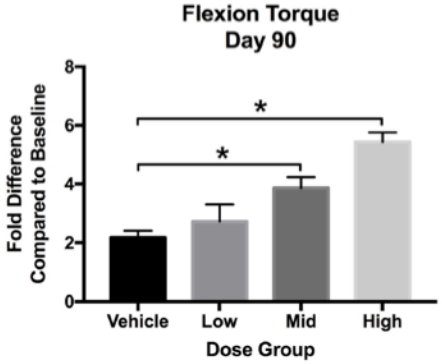
Durable

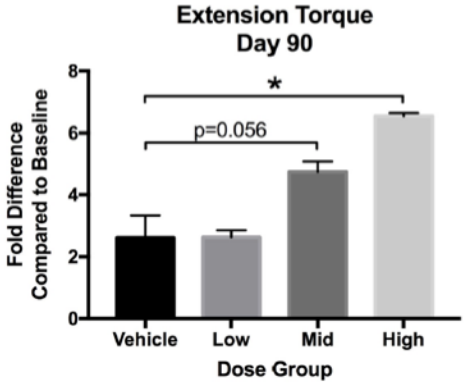


1 Month | 30 Months

Systemic administration of SGT-001 led to body-wide and sustained transgene expression for more than 30 months

Functional Benefit





N = 3 per cohort

Dose-dependent, sustained expression of microdystrophin correlated with statistically significant improvement in muscle function

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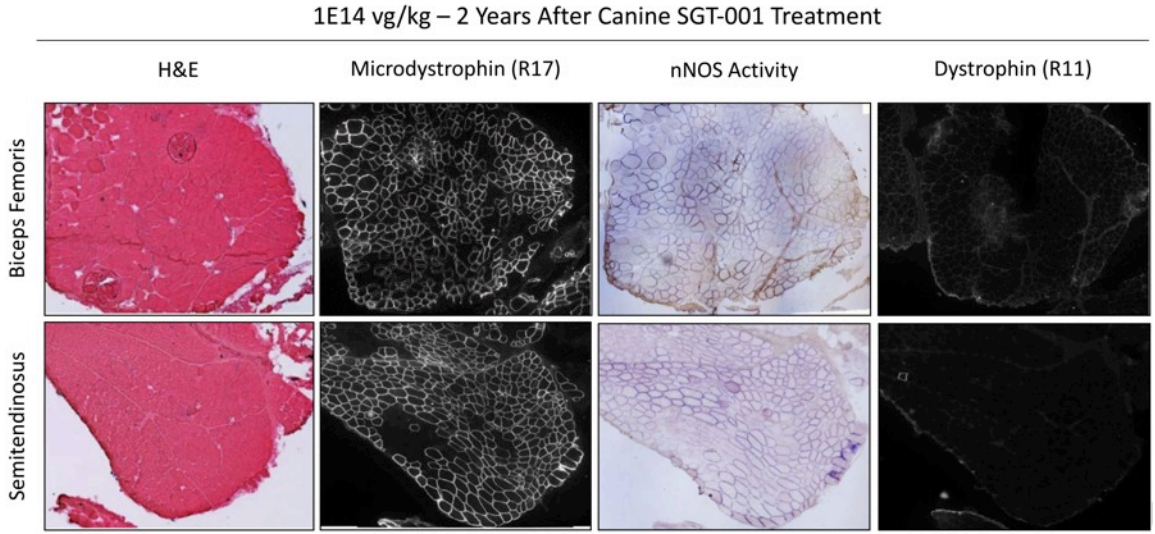
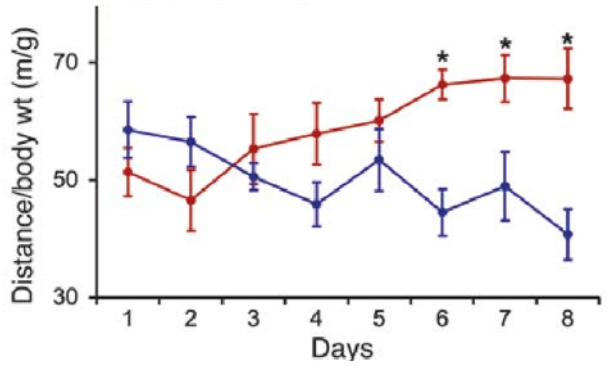
SGT-001 Restored Important Dystrophin Associated Proteins, Including nNOS

Neuronal Nitric Oxide Synthase (nNOS)-Restoring Microdystrophin Improved Muscle Function and Blood Circulation



The presence of microdystrophin correlates with neuronal nitric oxide synthesis in the muscle

Treadmill Endurance Assay In Mouse Model



nNOS Restoring Micro-gene

Non-nNOS Restoring Micro-gene

*Source: *Modified from Lai et al.* Dystrophins carrying spectrin-like repeats 16 and 17 anchor nNOS to the sarcolemma and enhance exercise performance in a mouse model of muscular dystrophy. *The Journal of Clinical Investigation 2009.*

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IGNITE DMD: Phase I/II Adaptive Clinical Trial

Randomized, controlled, open-label, single-ascending dose

Plan to enroll 16-32 ambulatory and non-ambulatory children and adolescents

IGNITE DMD clinical trial initiated; First patient dosed

Clinical hold lifted; activities to resume enrollment underway

Safety (primary endpoint)

Efficacy (primary endpoint)

- Microdystrophin expression

Efficacy (secondary endpoints)

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

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Recent IGNITE DMD Protocol Updates

- Modified steroid regimen, including IV glucocorticoids in initial weeks post administration
- Enhanced monitoring measures, including complement panel
- Eculizumab available if complement activation observed
- Dosing several children prior to dosing additional adolescents
- Option to obtain pre-specified intermediate biopsy at day 45

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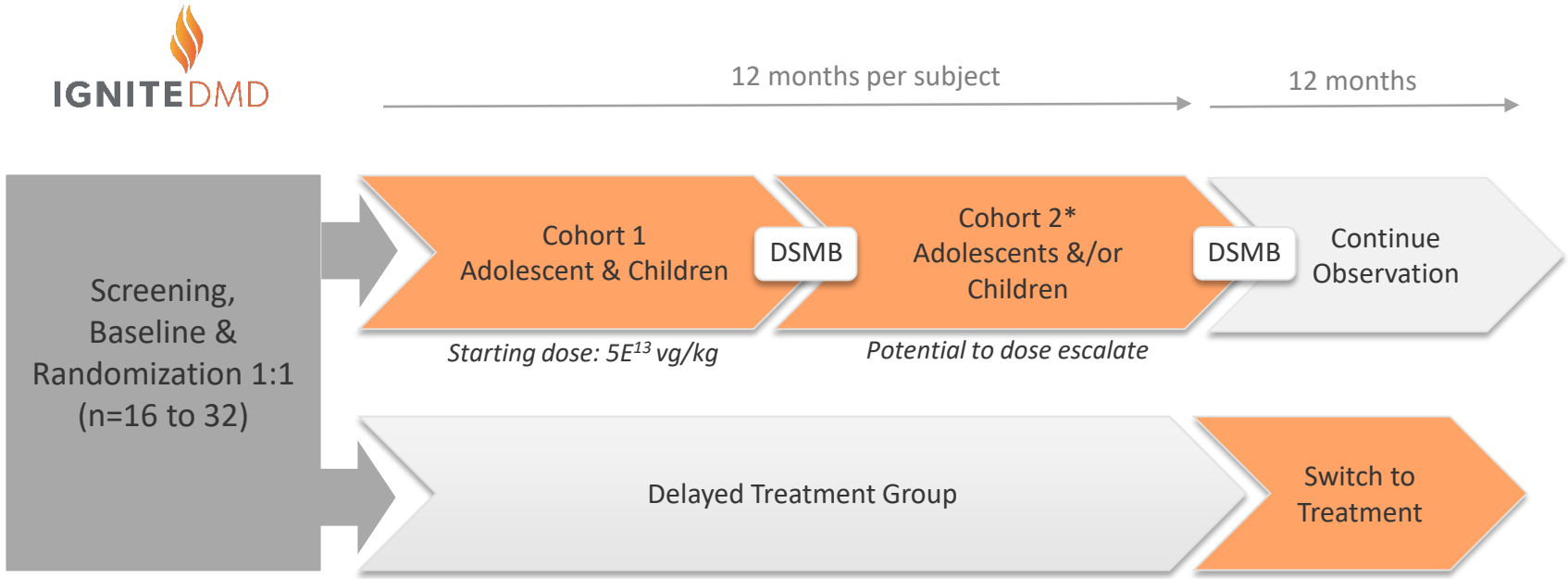
IGNITE DMD Clinical Trial Design



*The adaptive design of IGNITE DMD allows for more than two dose cohorts depending on safety data reviews.

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Scalable Manufacturing Process To Meet Clinical and Commercial Needs

Significant early investment to establish capabilities

- Hired top-tier process development team
- Built process development and characterization lab
- Established scalable AAV process in suspension in-house

Currently producing clinical trial supply

- Developed suspension method→ helps to ensure ability to supply high doses
- Partnered with two top-tier CMOs → ensures redundancy
- Secured dedicated CMO capacity → helps to ensure continuity of production

Preparing to meet commercial demand

- Increasing scale of AAV production; currently at 250L
- Improving specific productivity of process to increase yield
- Evaluating in-house manufacturing for long-term needs

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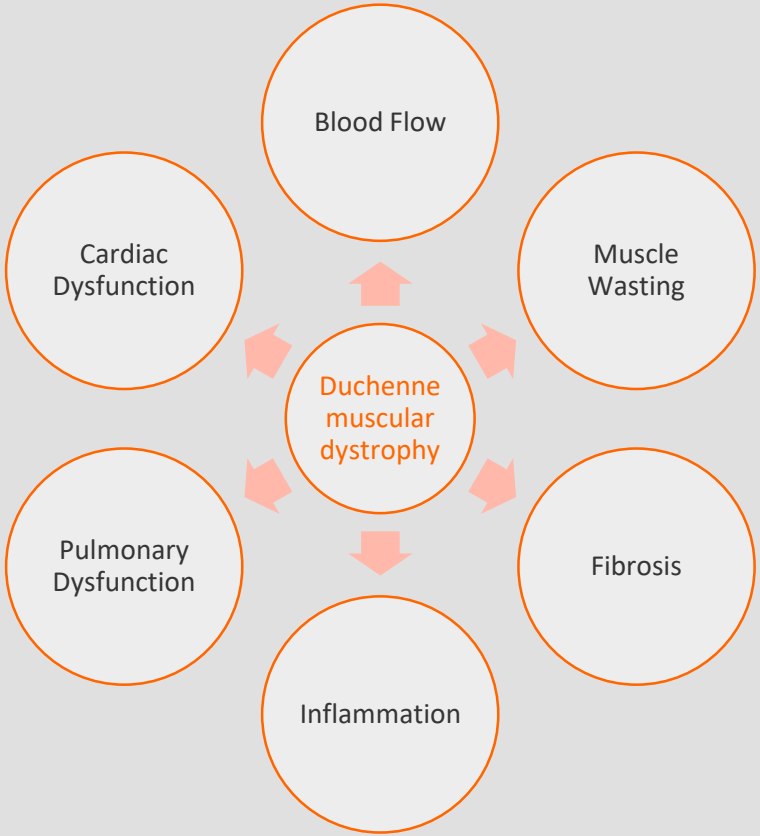
DISEASE MODIFYING THERAPIES

Addressing Symptoms

Significant Need For Additional Symptomatic Therapies



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SB-001: Lead Disease-Modifying Therapy

Anti-LTBP4 antibody

- LTBP4 expression is a powerful genetic modifier of DMD disease progression
- Compelling natural history data

Program goal

- Develop anti-LTBP4 antibody to block TGF-β activation
- Reduce fibrosis and inflammation

Strategic collaborations

- Elizabeth McNally, M.D., Ph.D., of Northwestern University
- Adimab

Next steps

- Develop in vitro assays for selection of lead candidates

Planning to initiate preclinical development in 2018

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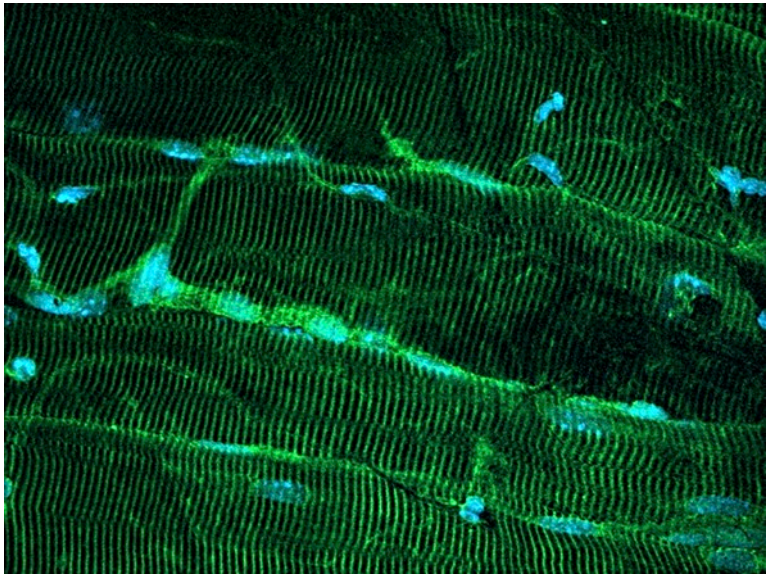
Program Based On Compelling Natural History Data

Mutations in the LTBP4 Gene Delayed the Loss of Ambulation In DMD Patients

Patients with LTBP4 modifier lose the ability to walk at an older age

The LTBP4 protein is a recognized genetic modifier of DMD. It is believed that LTBP4 plays a role in fibrosis and inflammation.

LTBP4 is Highly Expressed in Muscle Fibers



The green fibers represent LTBP4 expression

Source: Flanigan et al. LTBP4 Genotype Predicts Age of Ambulatory Loss in Duchenne Muscular Dystrophy. Annals of Neurology 2013.

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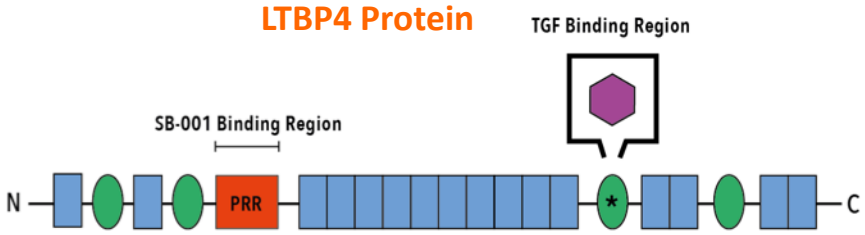
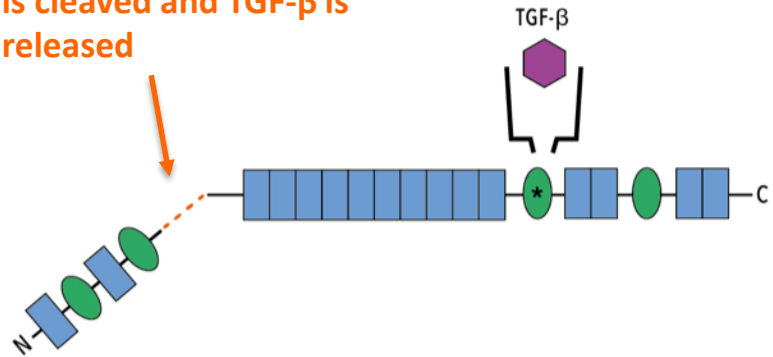
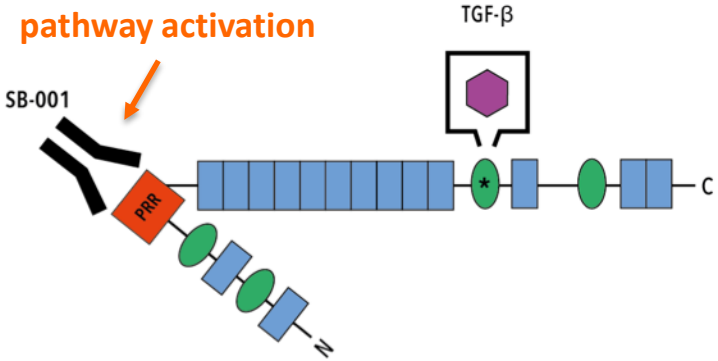
SB-001: An Anti-LTBP4 Antibody



The fibrosis pathway is activated when the PRR site is cleaved and TGF-β is released



SB-001 is designed to prevent cleavage and sequester release of TGF-β, preventing the fibrosis pathway activation



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Future Innovation

Future DMD Innovation

Understanding Underlying Disease

Biomarkers, Sensors, Improved Clinical Endpoints

Uncovering New Potential

Novel Mechanisms and Targets

Targeting Every Patient

Next Generation Promoters, Vectors and Transgenes

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Corporate

Experienced Leadership

Management Team

Ilan Ganot
President, Chief Executive Officer, Founder

Alvaro Amorrortu
Chief Operating Officer

Carl Morris, Ph.D.
Chief Scientific Officer

Jorge A. Quiroz, M.D.
Chief Medical Officer

Joel Schneider, Ph.D.
Chief Technology Officer, Head of Exploratory R&D

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Managing Director, RA Capital Management

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Financial Snapshot

$129.1 million
Net proceeds from IPO

$182.4 million
Cash, equivalents and available-for-sale securities at March 31, 2018

~35.5 million
Shares outstanding

Analyst coverage
JP Morgan, Goldman Sachs, Leerink, Nomura, Chardan

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Thank You

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