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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

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**FORM 8-K**

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**CURRENT REPORT  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of report (Date of earliest event reported): January 7, 2019**

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**Solid Biosciences Inc.**  
(Exact Name of Registrant as Specified in Charter)

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**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-38360**  
(Commission  
File Number)

**90-0943402**  
(IRS Employer  
Identification No.)

**141 Portland Street, Fifth Floor**  
**Cambridge, MA 02139**  
(Address of Principal Executive Offices) (Zip Code)

**Registrant's telephone number, including area code: (617) 337-4680**

(Former Name or Former Address, if Changed Since Last Report)

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Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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**Item 7.01. Regulation FD Disclosure.**

On January 9, 2019, Solid Biosciences Inc. (the "Company") intends to make a slide presentation at the 37th Annual J.P. Morgan Healthcare Conference. Beginning on January 8, 2019, the Company also plans to use the presentation in meetings with investors. A form of the slide presentation is being furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information responsive to Item 7.01 of this Form 8-K, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits:

[99.1 Form of Presentation of Solid Biosciences Inc., dated January 8, 2019](#)

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

SOLID BIOSCIENCES INC.

Date: January 7, 2019

By: /s/ Jennifer Ziolkowski

Name: Jennifer Ziolkowski

Title: Chief Financial Officer



# Solid Biosciences

Ilan Ganot, Co-Founder, CEO and President

January 2019

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.

# Purpose-Built to Solve Duchenne Muscular Dystrophy

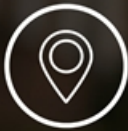


**360-degree Approach**  
Address all facets of DMD

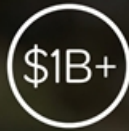
**Differentiated Lead Gene Transfer**  
Preliminary clinical data this quarter

**Scalable Manufacturing Process**  
Meet clinical and commercial needs

# Duchenne Is A Devastating Muscle-Wasting Disease



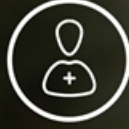
10-15,000 cases  
in the U.S.



Economic  
burden in U.S.\*



Progressive  
& irreversible



No good  
treatment options



Caused by mutations in the  
dystrophin gene



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



## CORRECTIVE THERAPIES



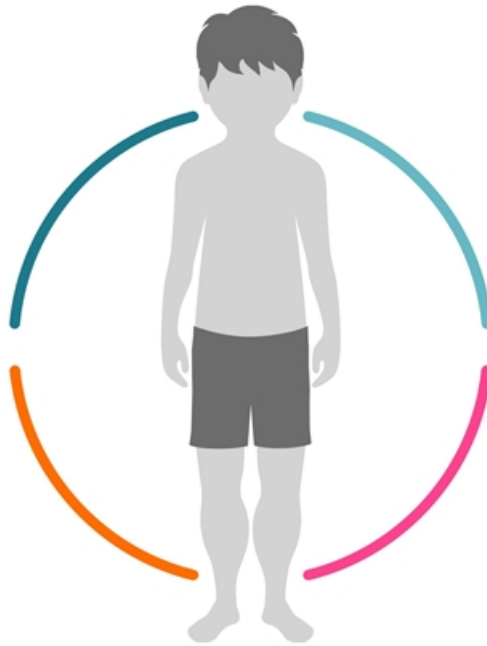
Gene therapy to address the genetic cause of DMD



## DISEASE UNDERSTANDING



Biomarkers and endpoints to improve development



## DISEASE-MODIFYING THERAPIES



Small molecules and biologics to address symptoms



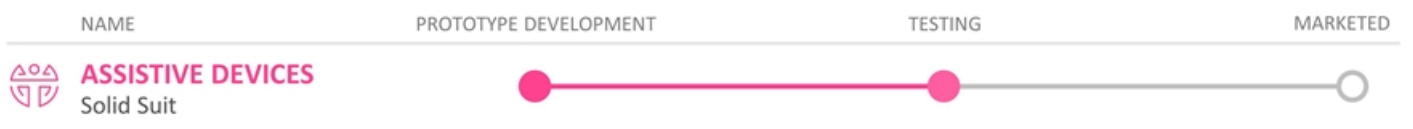
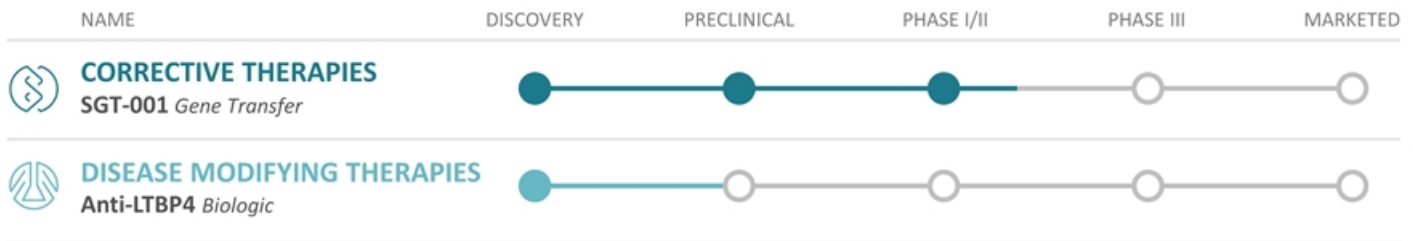
## ASSISTIVE DEVICES



Technology to support mobility



# Solid Pipeline





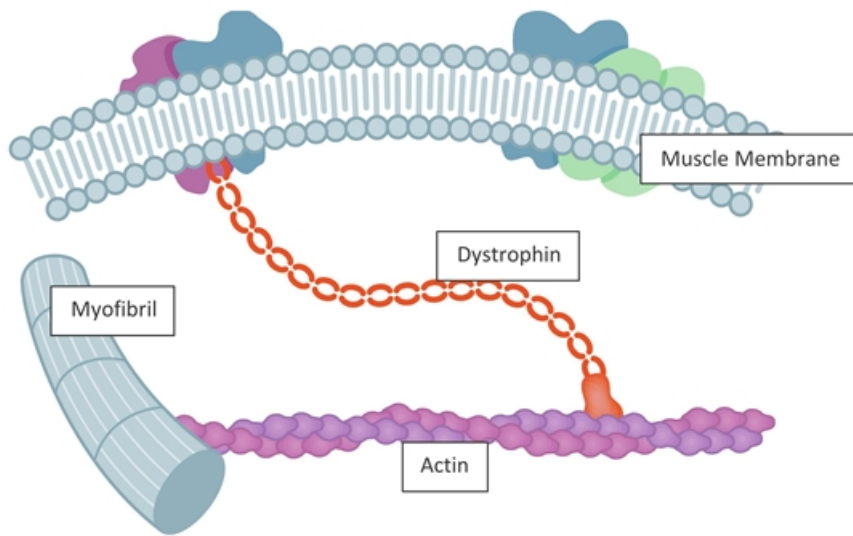
Corrective Therapies

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# Innovation in Gene Transfer

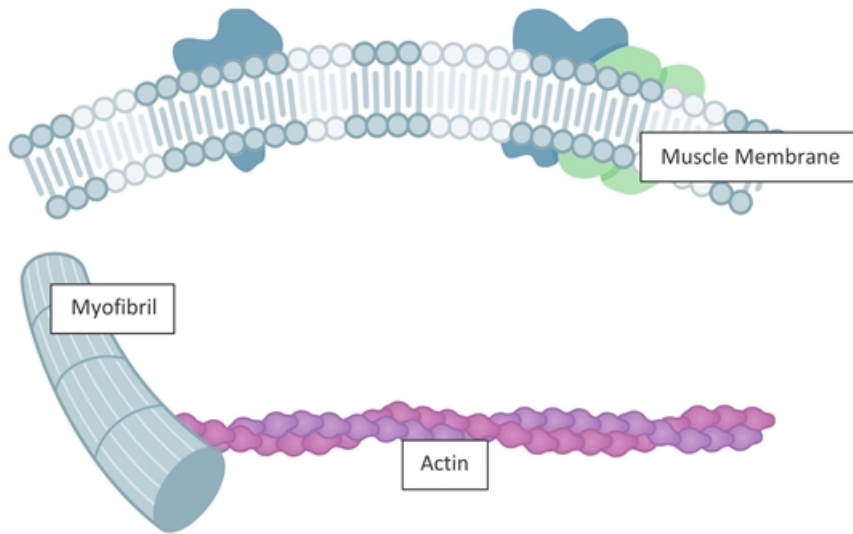


## HEALTHY MUSCLE

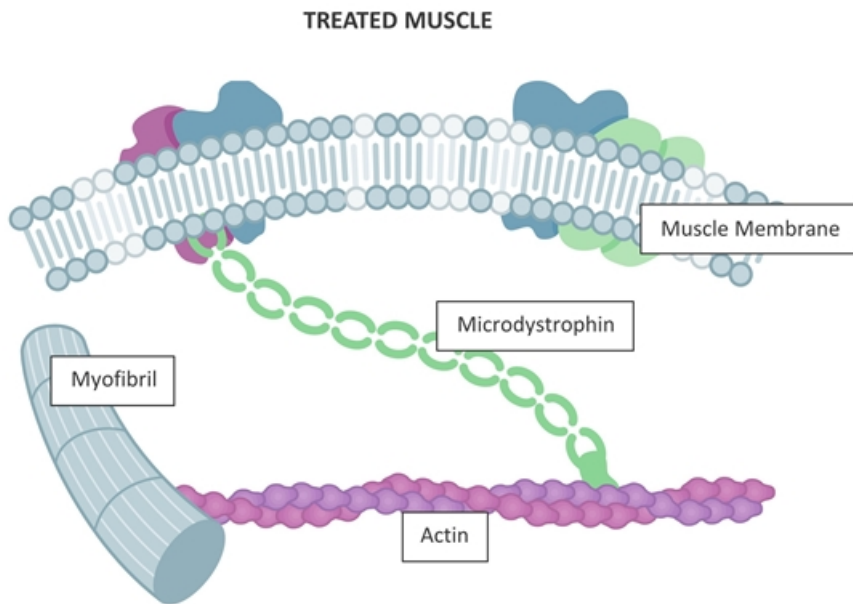


- Dystrophin protects the muscle from damage and stabilizes critical dystrophin-associated proteins

## DYSTROPHIC MUSCLE



- In DMD, mutations in the dystrophin gene result in the loss of functional dystrophin protein
- Muscle fibers become unstable, lose the ability to repair and become fibrotic



- Microdystrophin gene transfer encodes for a functional dystrophin protein surrogate designed to replace the missing dystrophin protein

# Each Component Of SGT-001 Was Carefully Selected



**Transgene**



Restore key functions  
of a complex protein



**SGT-001  
microdystrophin**



**Promoter**



Expression is highly targeted



**CK8**



**Vector**

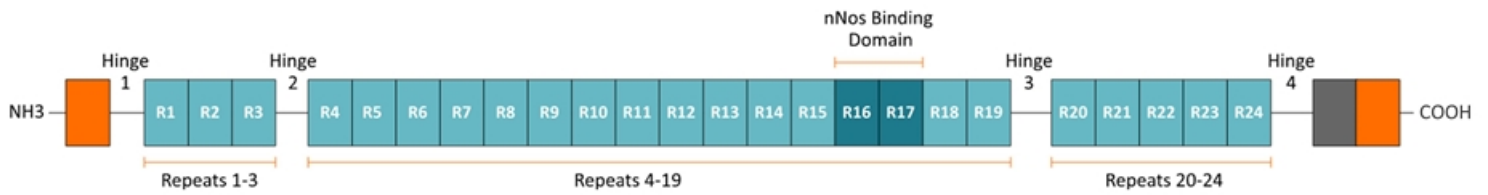


Skeletal and cardiac transduction

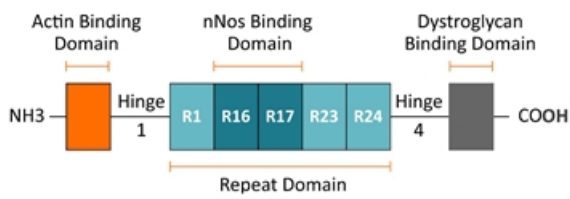


**AAV9**

## Full Length Dystrophin Protein



## SGT-001 Microdystrophin Protein

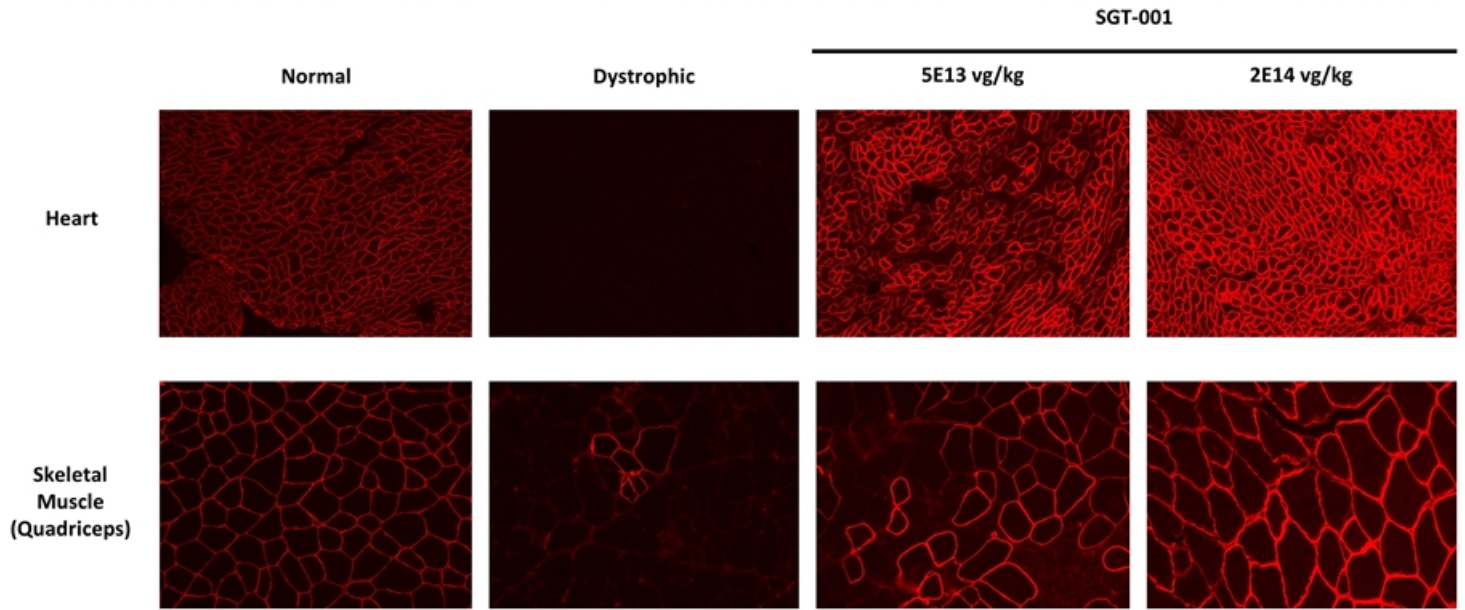


- Exclusive licenses to key patent portfolios covering microdystrophin variants and functional domains (e.g. the nNOS binding domain)
- SGT-001 selection based on more than 30 years of research; confirmed through internal comparative analysis

Visual representation only

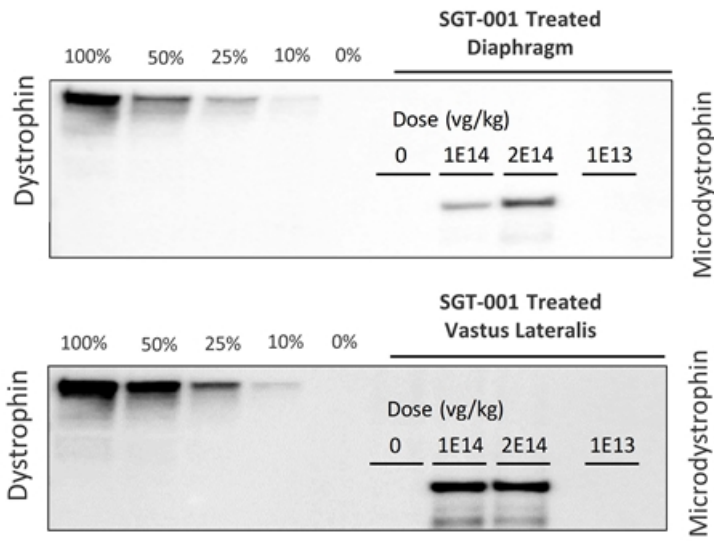


# SGT-001 Promotes Significant Cardiac And Skeletal Muscle Microdystrophin Expression In Mice

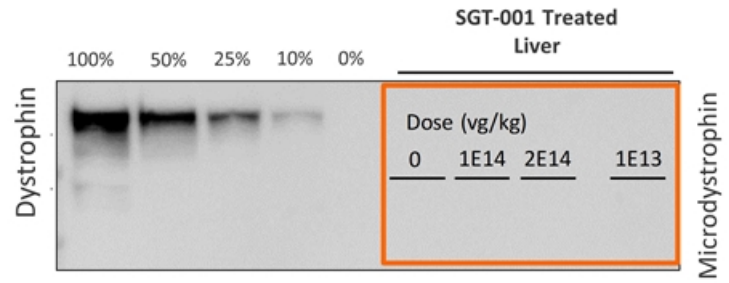


# CK8 Muscle-Specific Promoter Restricts Expression To Muscles

## Target Tissue

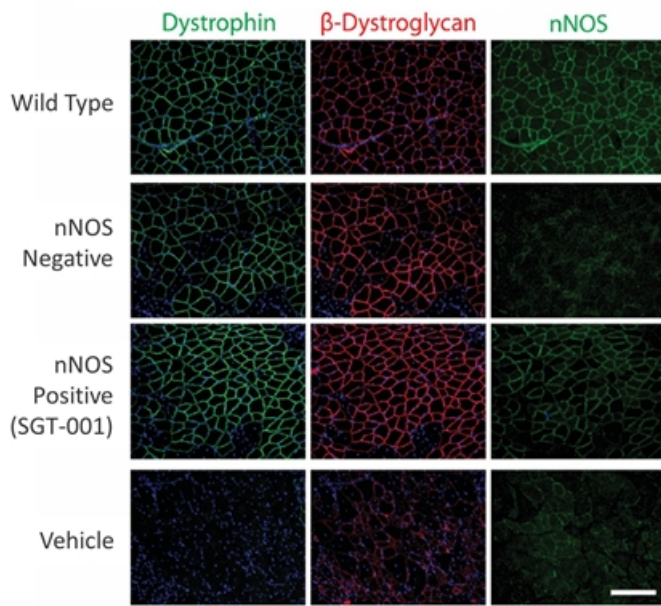


## Non-target Tissue

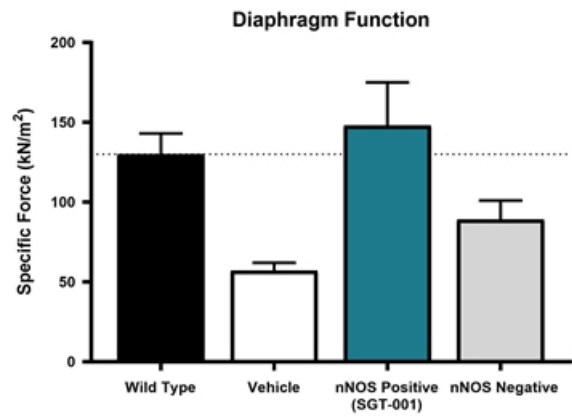


Solid Biosciences data on file. Three month efficacy study in GRMD canines. Representative only.

# Microdystrophin With nNOS Binding Domain Selected Based On Extensive Comparative Analysis

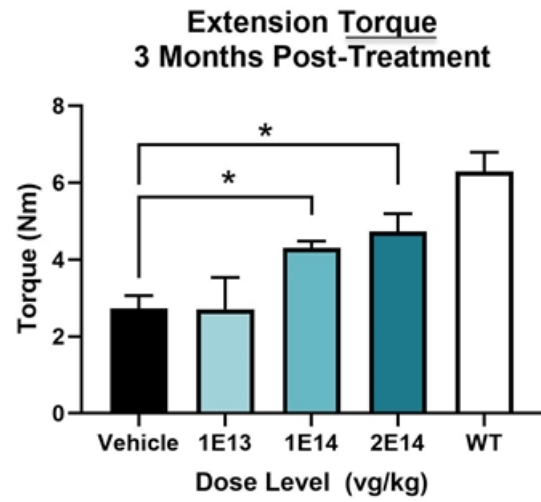
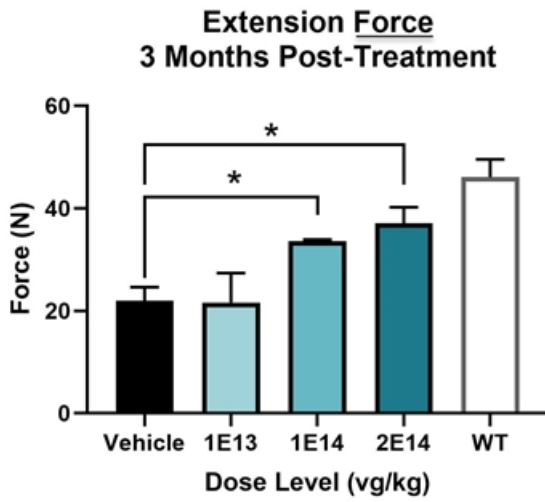


SGT-001 treatment led to force generation levels comparable to those in wild type mice



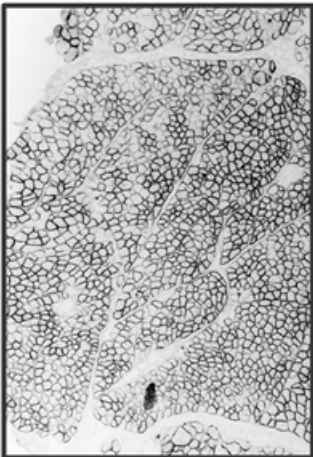
Specific diaphragm force 6 months post-treatment. Data shown as mean  $\pm$  SEM. n=5-7 per group.

# Significant Functional Benefit Demonstrated In Dystrophic Canines

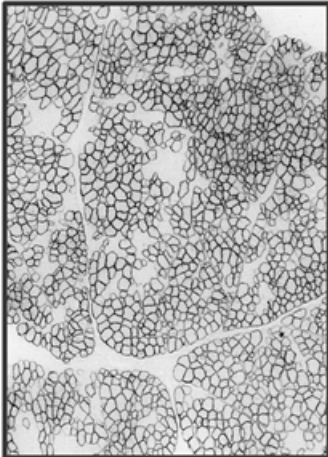


Solid Biosciences data on file. Three month efficacy study in GRMD canines. N=3 per group | \* p<0.05 statistical significance.

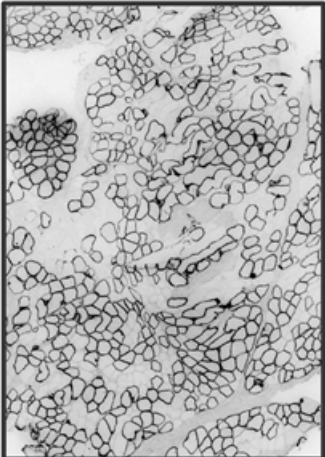
# Long-term Durability Observed In Canines



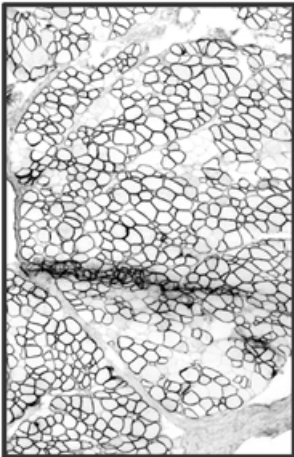
1 Month



3 Months



24 Months



30 Months

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

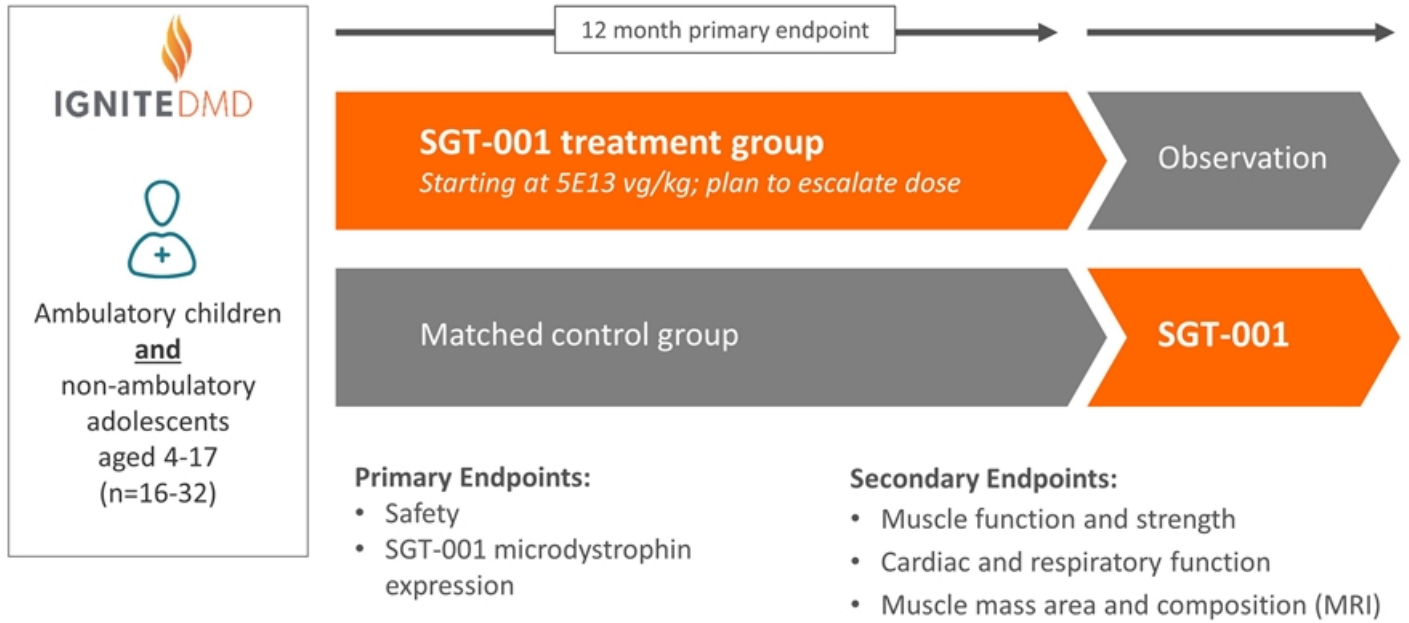


SGT-001 Clinical Program

IGNITE DMD









## Designed to obtain efficacy data at multiple dose levels

- Microdystrophin expression, muscle function and dose response

## Includes randomized control arm for comparative data

- Multiple patients and their matched controls enrolled

## Supports evaluation across a broad population

- Patients of different ages and stages of disease progression

## Offers flexibility to assess kinetics of microdystrophin expression over time

- Biopsies at baseline, 12 months and intermediate timepoint (45 days; 3, 6 or 9 months)

## Will enable productive conversations with regulators



## Preliminary data anticipated in Q1 2019

- ✓ Microdystrophin expression via western blot and immunofluorescence
- ✓ nNOS localization
- ✓  $\beta$ -Sarcoglycan localization
- ✓ Biodistribution
- ✓ Supportive data, as appropriate

Manufacturing

Producing Materials



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

## Solid Manufacturing Strategy

Move quickly with a process that scales up to meet the needs of all patients with DMD

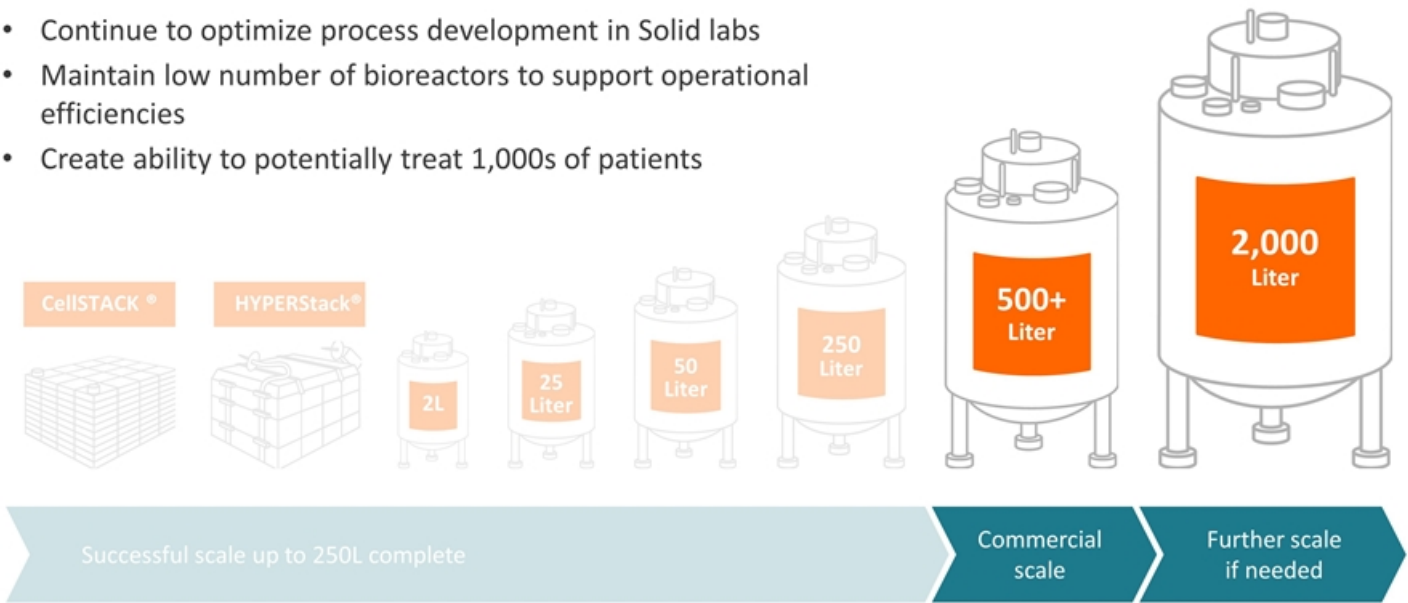
# GMP Manufacturing Process Currently Producing At Significant Volume

- Successfully scaled up to 250L in suspension and produced multiple batches
- Each 250L batch can dose multiple patients
- Utilizes proven, validated and widely-available standard bioreactors



# Scaling Process To Efficiently Supply Commercial Markets

- Continue to optimize process development in Solid labs
- Maintain low number of bioreactors to support operational efficiencies
- Create ability to potentially treat 1,000s of patients

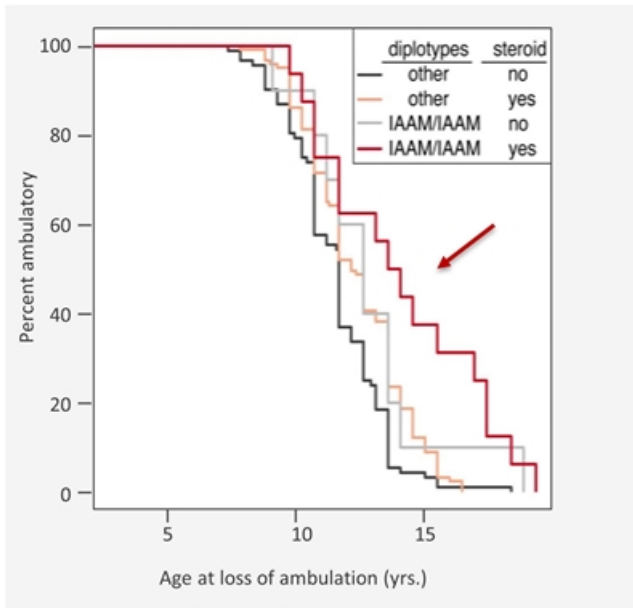


LTBP4 and Next Generation  
Gene Therapies

Expanding Pipeline

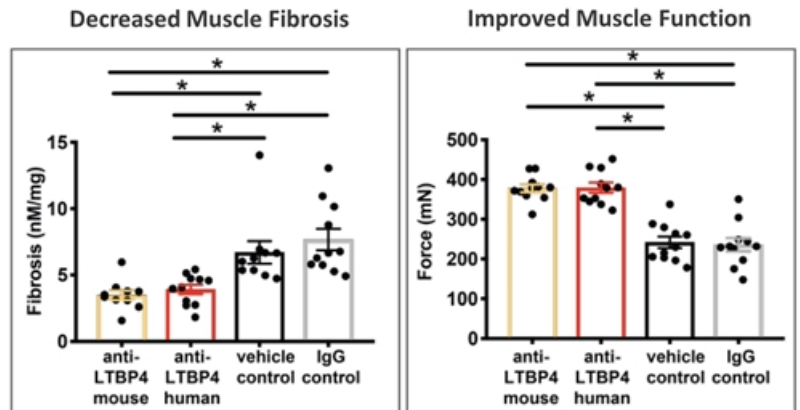


## LTBP4 is a powerful genetic modifier in DMD\*



\*Flanigan et al. *Annals of Neurology*. 2013.

## Positive results from blinded, 24-week efficacy study



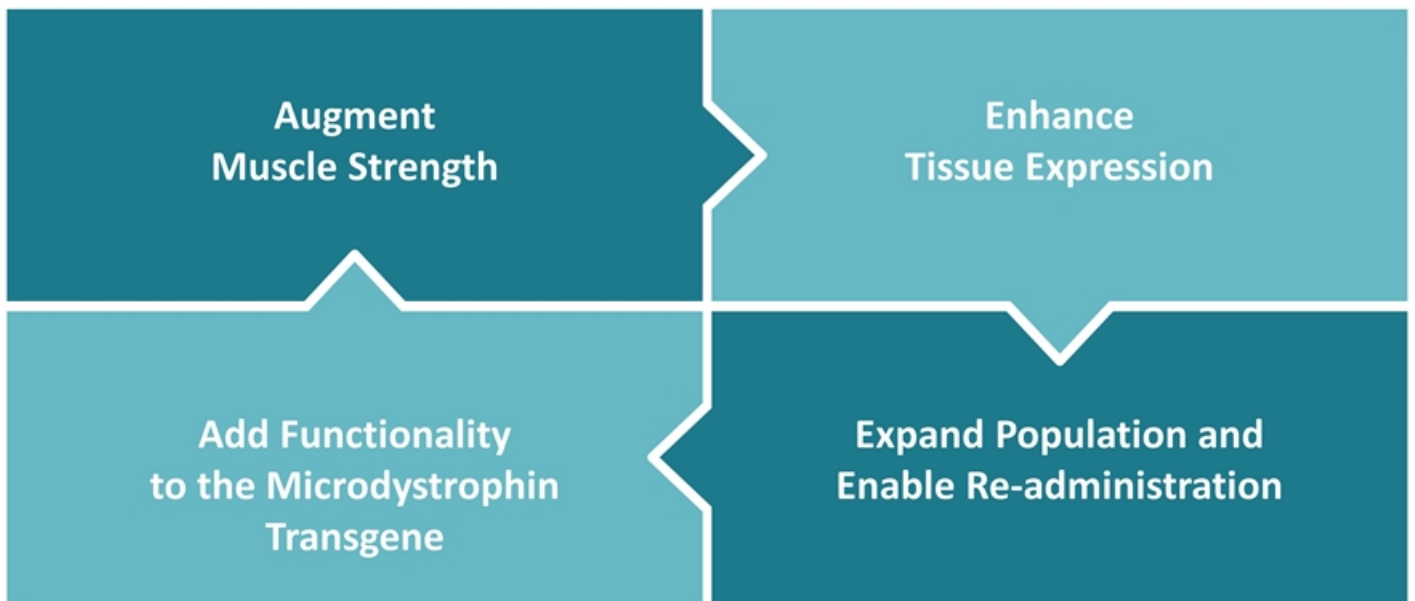
*mdx/hLTBP4 mice, dosed weekly x 24wks (Demonbreun, Quattrocelli, McNally – unpublished data)*

Ikaika Therapeutics

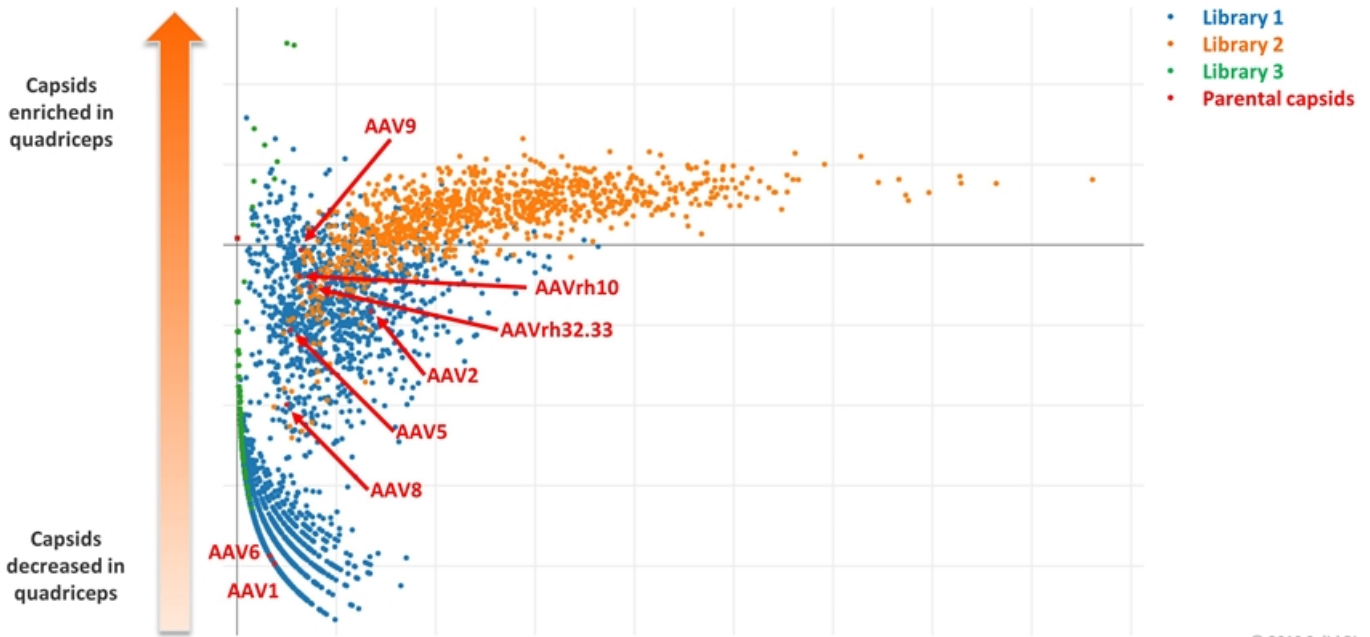
Northwestern Medicine  
Feinberg School of Medicine



# Internal And Partnered Programs To Build Comprehensive Pipeline For Duchenne



# Next Generation Screening in Disease-Specific Models Yields Novel Potential AAV Candidates



## SGT-001 Clinical Data

- Preliminary data in the first quarter of 2019
- Interim analysis in the second half of 2019

## Program Advancement

- Manufacturing process development and scale up
- Regulatory discussions to define approval path

## Pipeline

- Progress LTBP4 program toward IND
- Advance next generation promoters/vectors
- Support mission with targeted business development