



# Solid Biosciences

39<sup>th</sup> Annual J.P. Healthcare Conference

January 14, 2021

# Forward-Looking Statements

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This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the Company’s IGNITE DMD clinical trial, the safety or potential efficacy of SGT-001, potential milestone payments or royalty payments in connection with the Ultragenyx collaboration and other statements containing the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” “working” and similar expressions. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the Company’s ability to resume and/or continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities and investigational review boards at clinical trial sites; enroll patients in IGNITE DMD; continue to advance SGT-001 in clinical trials; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully optimize and scale its manufacturing process; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop DMD/Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary continue development of SGT-001, achieve its other business objectives and continue as a going concern. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company’s 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 the Company’s most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this presentation represent the Company’s views as of the date hereof and should not be relied upon as representing the Company’s views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company’s views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims 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.

# 2021 Priorities and Anticipated Milestones

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**Resume dosing patients  
in IGNITE DMD  
(Q1 2021)**

**Present 12-month safety  
& efficacy for patients 1-6  
(Q1 2021)**

**Present 90-day biopsy data  
for additional patients  
dosed in IGNITE DMD  
(2H 2021)**

**Advance towards  
commercial readiness**

**Prepare for registration  
study**

**Further pipeline  
expansion**



Shaped  
*by the*  
**Past**



Inspired  
*by the*  
**Present**



Driven  
*for the*  
**Future**

.....

Years of Experience  
Leading up to  
Today

*Shaped  
by our Personal  
Connection*

# Honoring Michael Counterman (7.11.1992-12.31.2020)



# Solid's Focus: Duchenne Muscular Dystrophy

## DMD FACTS



Caused by mutations in the dystrophin gene



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



Progressive & irreversible



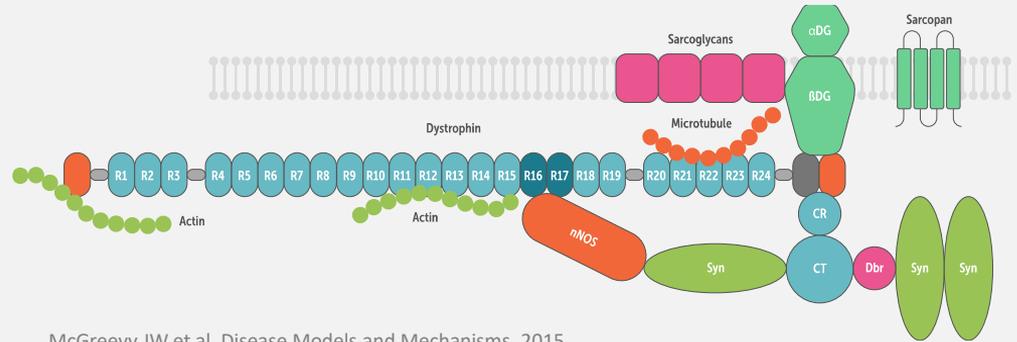
No good treatment options

- Founded in 2012
- Deep personal connection to and exclusive focus on Duchenne
- Employees with expertise from all corners of the Duchenne community
- Core technology portfolio covering a critical domain of dystrophin function

# SGT-001: Differentiated Microdystrophin Biology

## Dystrophin and the Glycoprotein Complex

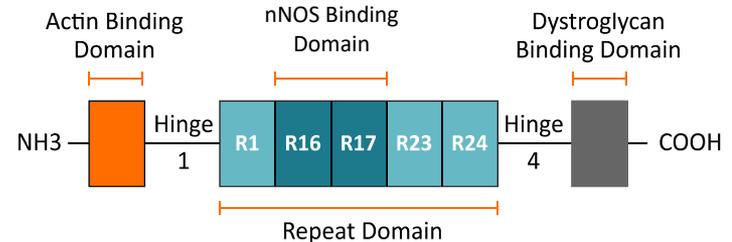
- Stabilizes the muscle membrane
- Acts as a molecular shock absorber
- Prevents muscle tissue damage and death
- Absent in Duchenne muscular dystrophy (DMD)



## SGT-001 Microdystrophin

- Microdystrophin is a rationally designed recombinant protein
- Able to be packaged into an AAV vector
- **Uniquely includes the nNOS\* binding domain**
  - Important for prevention of activity-induced ischemia and associated muscle injury
  - Presence correlated with milder phenotypes of Becker muscular dystrophy (BMD)
- Acts as a functional surrogate of full-length dystrophin

## SGT-001: Retains key dystrophin protein functional domains



\*nNOS: neuronal nitric oxide synthase

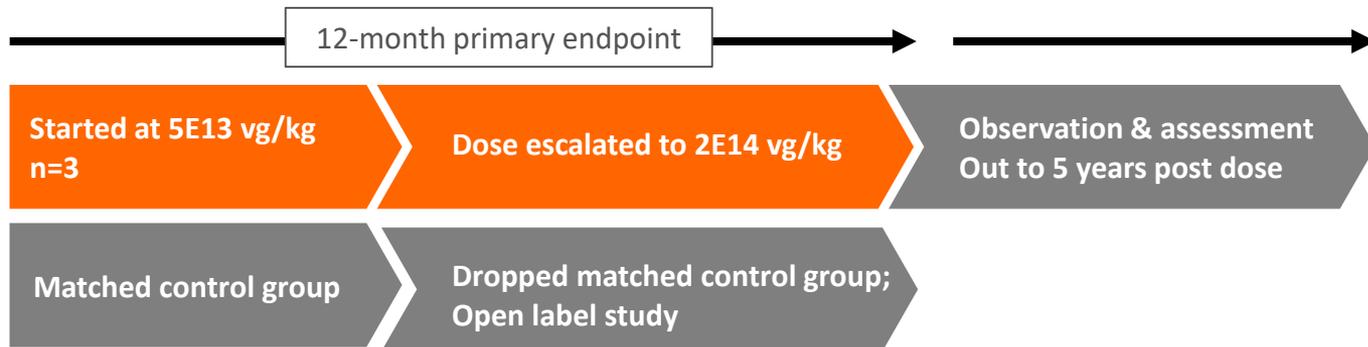
# IGNITE DMD Clinical Trial



**IGNITE**DMD



Ambulatory  
children  
Mutation  
agnostic



## Primary Endpoints:

- Safety
- SGT-001 microdystrophin expression at 12 months

## Secondary Endpoints:

- Muscle function and strength
- Cardiac and respiratory function
- Muscle mass area and composition (MRI)
- Patient and caregiver assessments of changes in functioning; quality of life

***6 Patients dosed; 5E13 vg/kg (n=3) 2E14 vg/kg (n=3)***

***Cleared in Q4 2020 to resume dosing in IGNITE DMD***

***All SAEs fully resolved; no additional drug related AEs***

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2020 – A Transformative Year

Inspired  
by the  
Present

## Clinical Protocol Amendments Made in Consult with FDA and Experts in Immunology and Complement Biology

### Designed to Inhibit Complement Activation

Give eculizumab and C1 esterase inhibitor prophylactically

### Dampening Immune Response

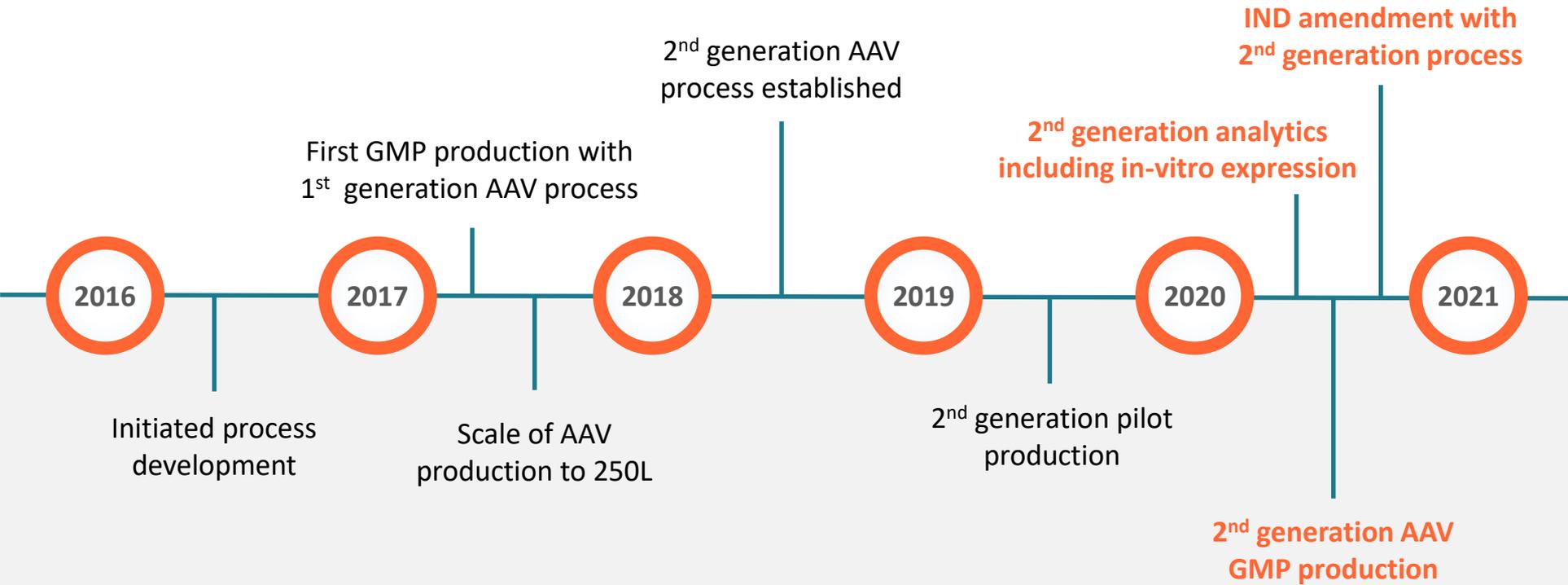
Transient increase in steroid regimen from 1 mg/kg to 2 mg/kg

### Reduce Total Viral Load (TVL)

18 kg weight limitation on next two patients\*

\*Resume dosing in smaller patients to decrease TVL; adjust weight upwards as appropriate

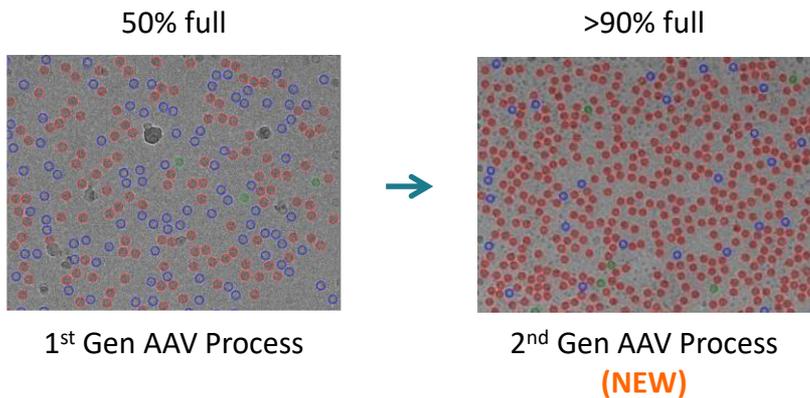
# Significant Investment in HSV Based AAV Manufacturing Platform



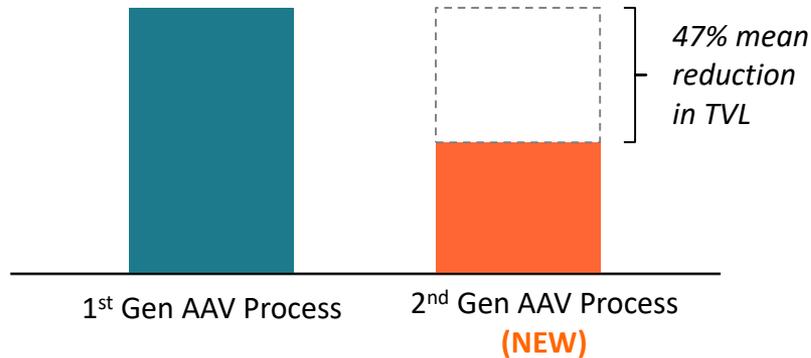
# 2<sup>nd</sup> Generation Manufacturing Process

Improvement from 50% to 90% full capsids in final drug product

## % of Full vs Empty Capsids



## Total Viral Load at 2e14 vg/kg



Reduced total viral load expected to improve dosing safety of SGT-001

# Encouraging 90-day Biopsy Results

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## Data from Subjects 4-6 Dosed at 2e14 vg/kg

- Higher of two doses evaluated in IGNITE DMD

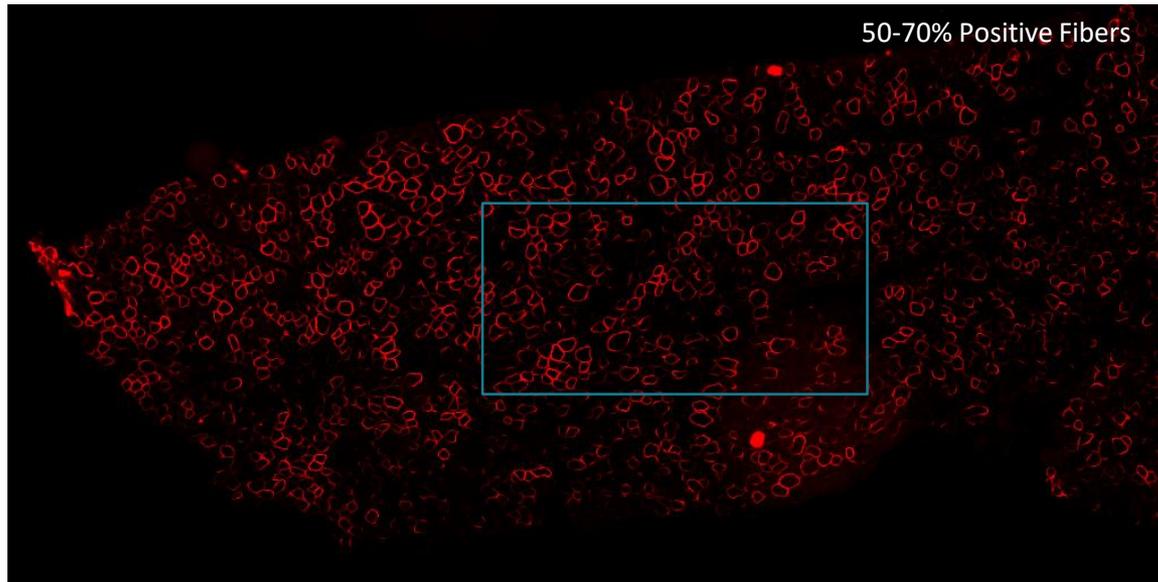


## Preliminary data showed that this dose has multiple potential beneficial effects:

- Muscle-wide distribution
- Membrane localization & stabilization of the Dystrophin Glycoprotein Complex
- Microdystrophin expression

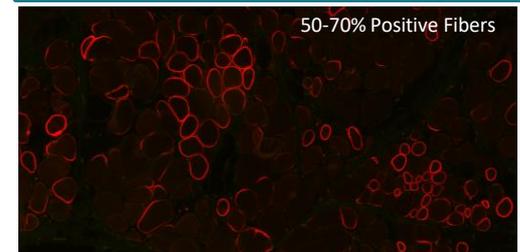
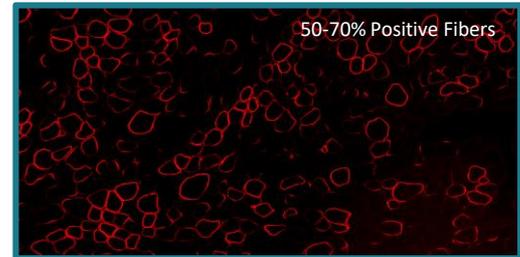
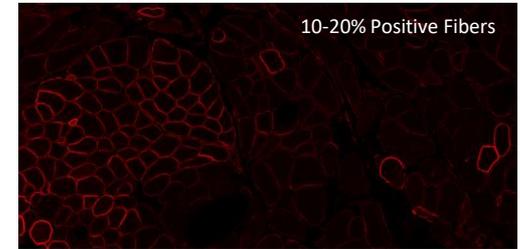
# Muscle-Wide Microdystrophin Expression at Day 90 in 2E14vg/kg Subjects

Low magnification image shows widespread distribution of microdystrophin positive fibers



20-70% Microdystrophin positive fibers observed in 2E14 vg/kg subjects

High magnification images of all three subjects



# Microdystrophin Stabilizes the DGC and nNOS at the Muscle Membrane

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Microdystrophin

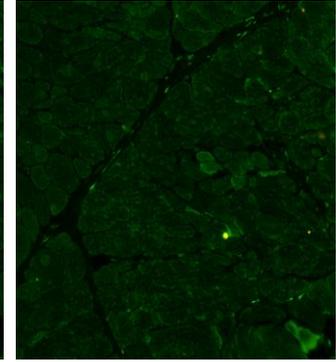
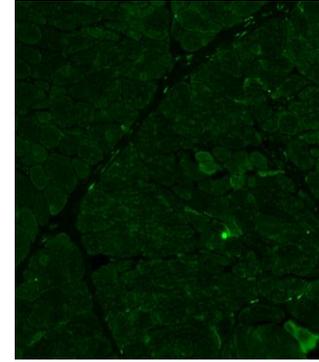
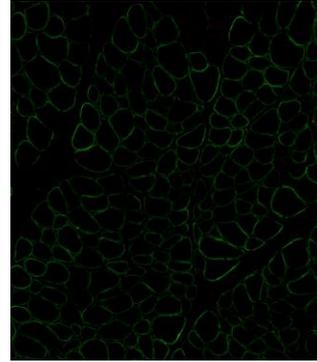
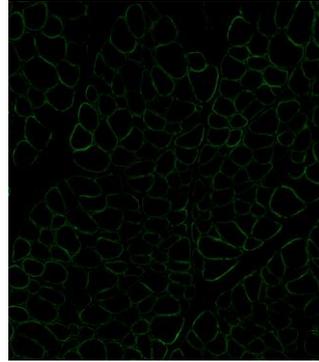
$\beta$ -Sarcoglycan

Merged

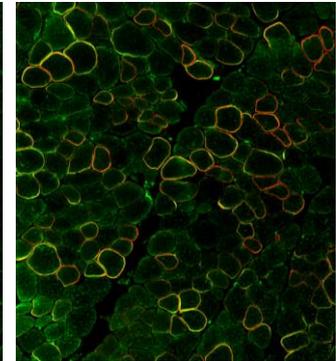
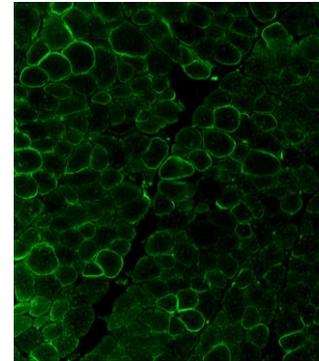
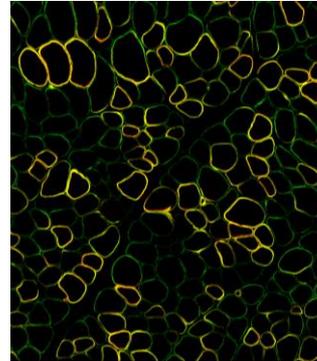
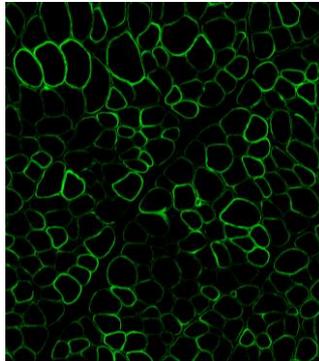
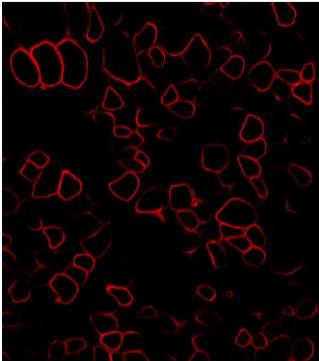
nNOS

Merged

Baseline



Day 90

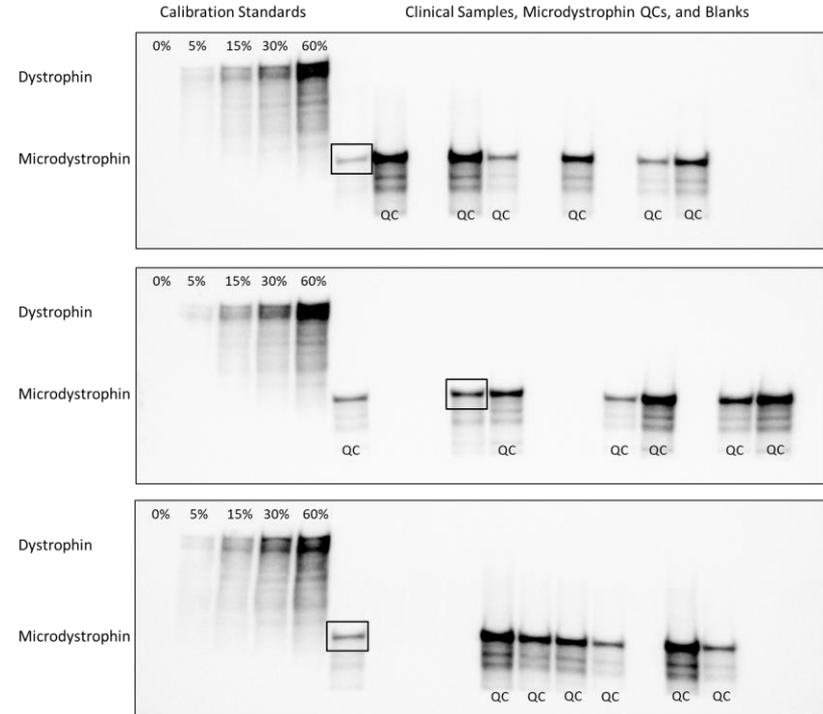


# Microdystrophin Protein at Day 90 Quantified by Western Blot

- Qualified Western blot assay
- Microdystrophin QCs run on each blot
- Day 90 protein levels ranged from ~5% to 17.5% of normal dystrophin

**Average Microdystrophin protein level  
~10% of normal dystrophin**

## Western Blots of 2E14 vg/kg Cohort Subjects



\*Boxed bands indicate clinical samples

# Collaboration to Advance Next Generation DMD Constructs

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- Proprietary nNOS-binding form of microdystrophin
- World class expertise in Duchenne and muscle biology



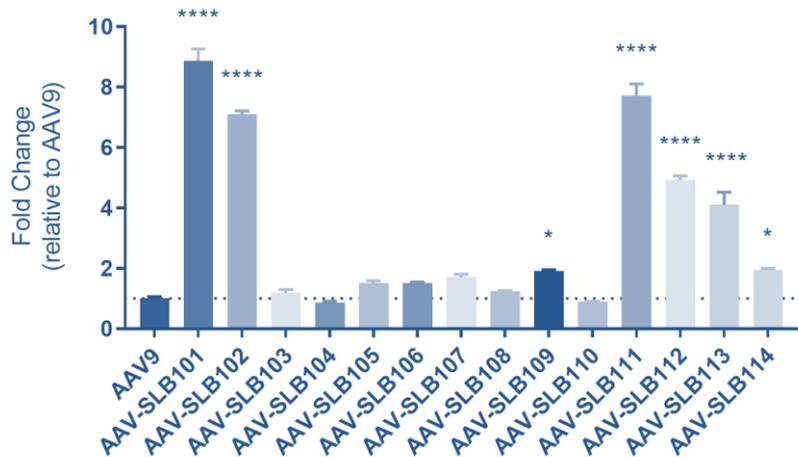
- HeLa PCL Platform: Commercial-grade 2,000L manufacturing capability
- AAV8 Variant with favorable immune profile

Solid retains exclusive rights to use of its microdystrophin in SGT-001, outside of AAV8 and other clade E variants.

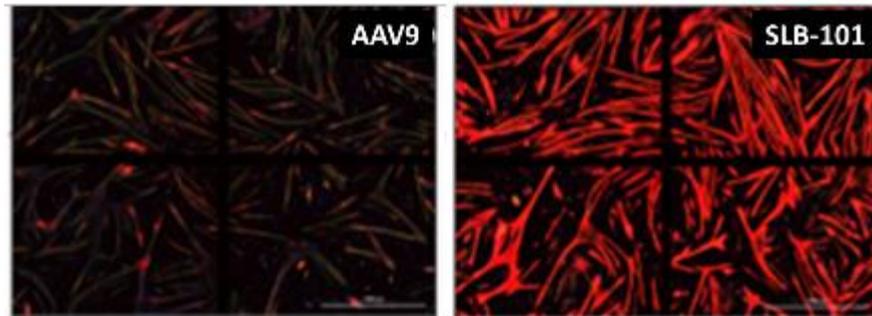
Solid received \$40M upfront through the sale of equity; Up to \$255 million in milestones plus royalty payments; option to opt-in to profit share at clinical proof of concept (POC)

# SLB-101: Next Generation Capsid Designed to Enhance Muscle Transduction Efficiency

## In vitro evaluation of novel, proprietary AAV-based candidate capsids



## Microdystrophin Expression in *in vitro* muscle cells



SLB-101 showed ~9-fold increased microdystrophin expression

***Continue to invest in next generation delivery technologies; enhancing muscle distribution***

# Financial Position Fortified on the Back of 2020 Achievements

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## \$150+ Million

OF CAPITAL RAISED IN 4Q2020

**\$40M**

Equity purchased  
at a 33% premium  
by Ultragenyx

**\$23.9M**

Gross proceeds of  
at-the-market sales  
in October 2020

**\$90M**

Gross proceeds from  
PIPE in December  
2020

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Significant  
Opportunities  
for Value  
Creation in 2021  
and Beyond

Driven  
for the  
Future



Shaped  
*by the*  
**Past**



Inspired  
*by the*  
**Present**



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Thank You

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