Solid Biosciences

Corporate Overview

November 2020

Forward-Looking Statements

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 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, to resume dosing in the IGNITE DMD trial, 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.

Overview & Strategy

- Singularly focused on developing transformative treatments for Duchenne muscular dystrophy
- Addressing the disease at its core by correcting the underlying mutation with our innovative gene therapy candidate SGT-001
- Initial biomarker patient data demonstrates that SGT-001 is biologically active, with differentiated properties that may result in unique clinical benefits

Duchenne Is A Devastating Muscle-Wasting Disease

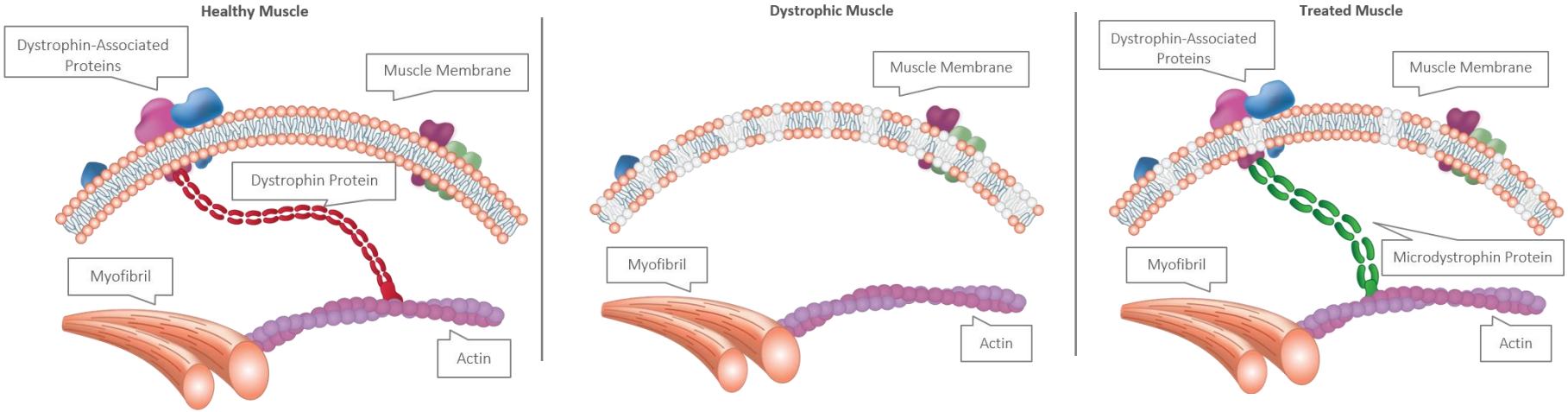
- 10-15,000 cases in the U.S.
- $1B+ Economic burden in U.S.*
- 100% FATAL Progressive & irreversible
- No good treatment options
- Caused by mutations in the dystrophin gene

*Landfeldt et al. Neurology, 2014.

Corrective Therapies

Innovation in Gene Transfer

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



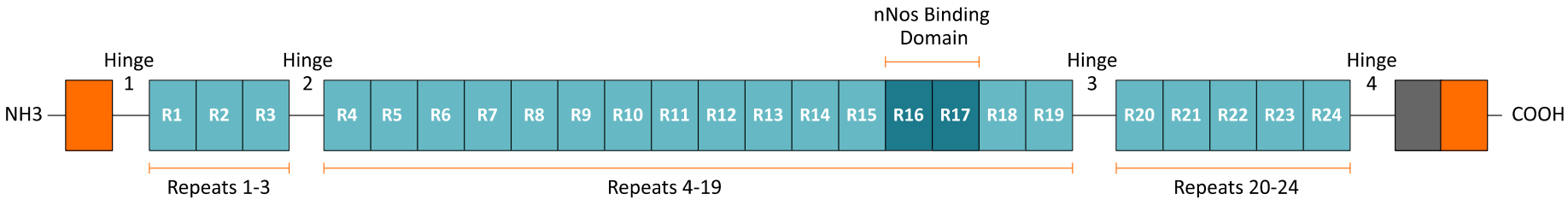
Visual representation only

Each Component Of SGT-001 Was Carefully Selected

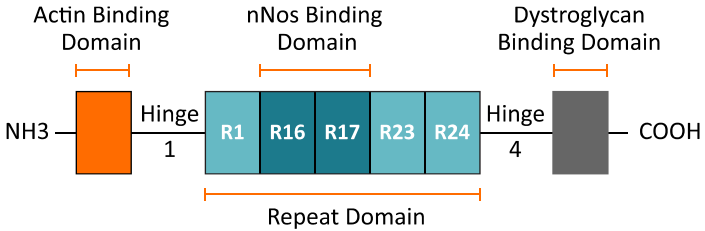
	**Transgene**	→	Restore key functions of a complex protein	→ **SGT-001 microdystrophin**
	**Promoter**	→	Expression is highly targeted	→ **CK8**
	**Capsid**	→	Skeletal and cardiac transduction	→ **AAV9**

SGT-001 Microdystrophin Has A Differentiated Composition

Full Length Dystrophin Protein



SGT-001 Microdystrophin Protein

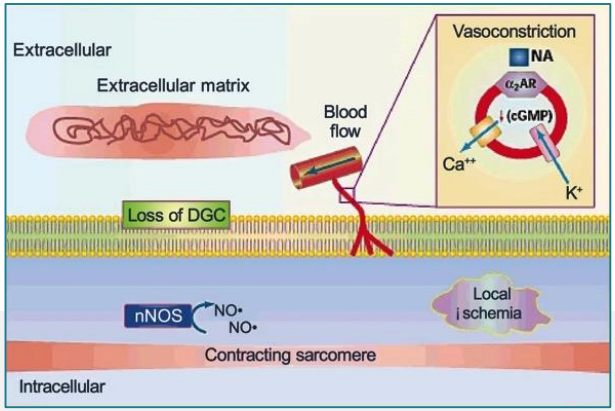


- Exclusive licenses to key patent portfolios covering microdystrophin variants and functional domains (e.g., the neuronal nitric oxide synthase (nNOS) binding domain)
- SGT-001 selection based on more than 30 years of research; confirmed through internal comparative analysis

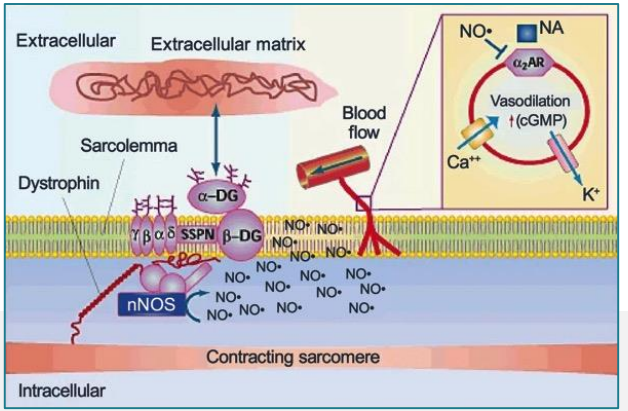
Visual representation only.

Importance of nNOS in Muscle

Lack of the nNOS domain of dystrophin prevents muscles from responding to high energy demand, such as exercise



Expression of the nNOS domain allows for muscles to receive appropriate blood flow when necessary to meet energy demands



- In Duchenne muscular dystrophy (DMD), the lack of membrane associated nNOS results in the inability of muscles to meet energy demands, causing muscle fiber turnover and development of fibrosis
- Although Becker muscular dystrophy (BMD) patients lack the majority of dystrophin, those who express the nNOS domain have been shown to produce the NO signaling molecule and demonstrate increased muscle function compared to those who do not
- Inclusion of this nNOS coding region of the dystrophin protein may result in microdystrophin protein that has unique activity, potentially providing important functional benefits such as diminished muscle fatigue and protection against ischemic muscle damage

Source: Crosbie R. NO vascular control in Duchenne muscular dystrophy. Nature Medicine (2001) 7:1; Boehler JF et. al. Membrane recruitment of nNOSμ in microdystrophin gene transfer to enhance durability. Neuromuscular Disorders (2019) 29: 735-741; Ramos JN et. al. Development of Novel Micro-dystrophins with Enhanced Functionality. Molecular Therapy (2019) 27, 3: 623-635

Third Quarter 2020 Updates

IGNITE DMD Clinical Hold Lifted

Modifications to IGNITE DMD trial protocol and improvements to manufacturing processes enable continued program development

- ✓ Implemented manufacturing process changes that remove the majority of empty viral capsids, allowing target dosing to be achieved with fewer viral particles.
- ✓ Provided the FDA information on new, quantitative, *in vitro* microdystrophin expression assay that demonstrates comparability between SGT-001 manufactured by the two processes.
- ✓ Continuing dosing at the 2E14 vg/kg dose.
- ✓ Reducing maximum weight of the next two patients dosed to 18 kg per patient, with potential weight increase in subsequent patients.
- ✓ Provided the FDA with updated safety and functional efficacy data for all patients dosed to date in IGNITE DMD.
- ✓ Amended the IGNITE DMD clinical protocol to include prophylactic use of complement inhibitor eculizumab and C1 esterase inhibitor and increasing the prednisone dose in the first month post dosing.

Plan to resume dosing in Q1 2021

Strategic Collaboration

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- Best-In-Class Microdystrophin
  - nNOS binding domain
  - Functional benefit in preclinical models
- World class expertise in Duchenne and muscle biology
- Solid retains exclusive rights to all other uses of its microdystrophin, including its existing SGT-001 program
- Solid received $40 million upfront through the sale of equity at a premium; up to $255 million in milestones plus royalty payments

ultragenyx pharmaceutical

- HeLa PCL Platform
  - Commercial-grade 2,000L large-scale manufacturing
- AAV8 Variant
  - Demonstrated favorable immune profile clinic
- Demonstrated track record of success in developing and commercializing innovative therapies for rare diseases

Development and commercialization of new gene therapies for Duchenne

Strengthened Balance Sheet

$40M

Strategic collaboration to develop and commercialize new gene therapies for Duchenne. Ultragenyx made a $40 million investment in Solid at a premium*

$23.9M

Shares of common stock sold through Jefferies LLC resulting in gross proceeds of $23.9 million

2H21

Cash and cash equivalents enable Solid to fund its operating expenses into the second half of 2021

**Ultragenyx has also agreed to pay up to $255 million in cumulative milestone payments per product upon achievement of specified milestone events, and tiered royalties on worldwide net sales. Upon achievement of proof-of-concept, Solid has the right to opt-in to co-fund collaboration programs in return for participation in a profit share or increased royalty payments.*

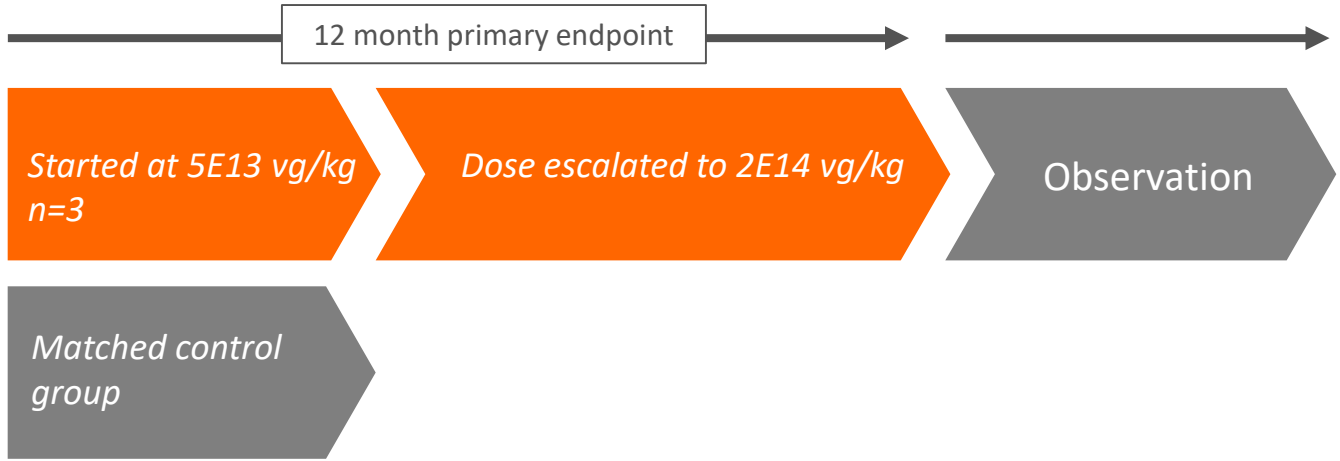
SGT-001 Clinical Program

IGNITE DMD

SGT-001 Phase I/II Clinical Study

IGNITEDMD

Ambulatory children
Mutation agnostic



Primary Endpoints:

- Safety
- SGT-001 microdystrophin expression at 12 months

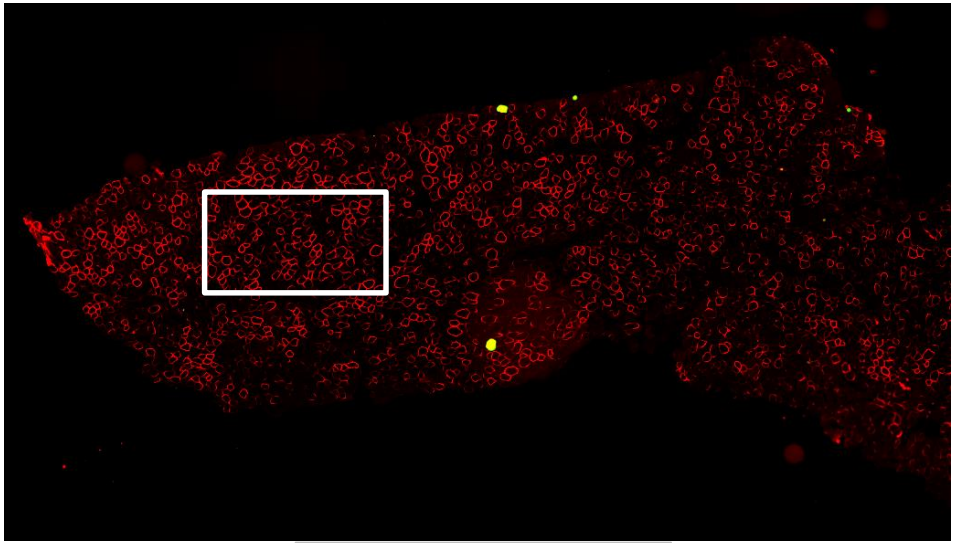
Secondary Endpoints:

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

Note: Solid is reducing the maximum weight of the next two patients dosed to 18kg

SGT-001 Administration to DMD Patients in 2E14 vg/kg Dose Cohort Resulted in Dose-Dependent, Muscle-Wide Microdystrophin Expression

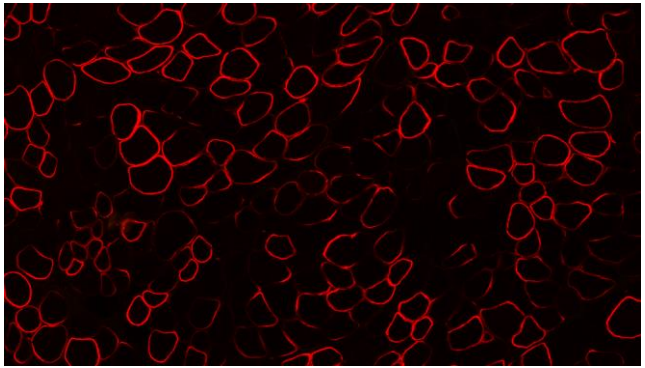
Low magnification image shows widespread microdystrophin positive fibers

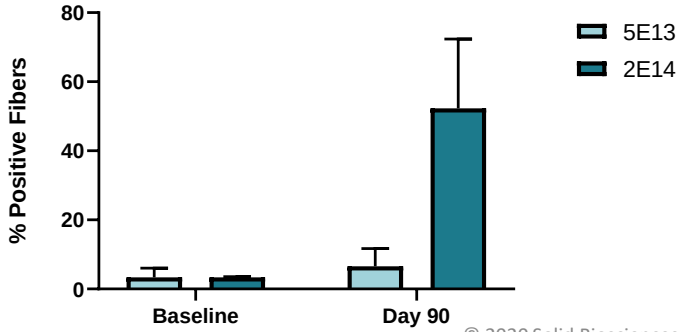


RED	Microdystrophin
GREEN	Revertant Fibers

Representative Image – 2E14 vg/kg

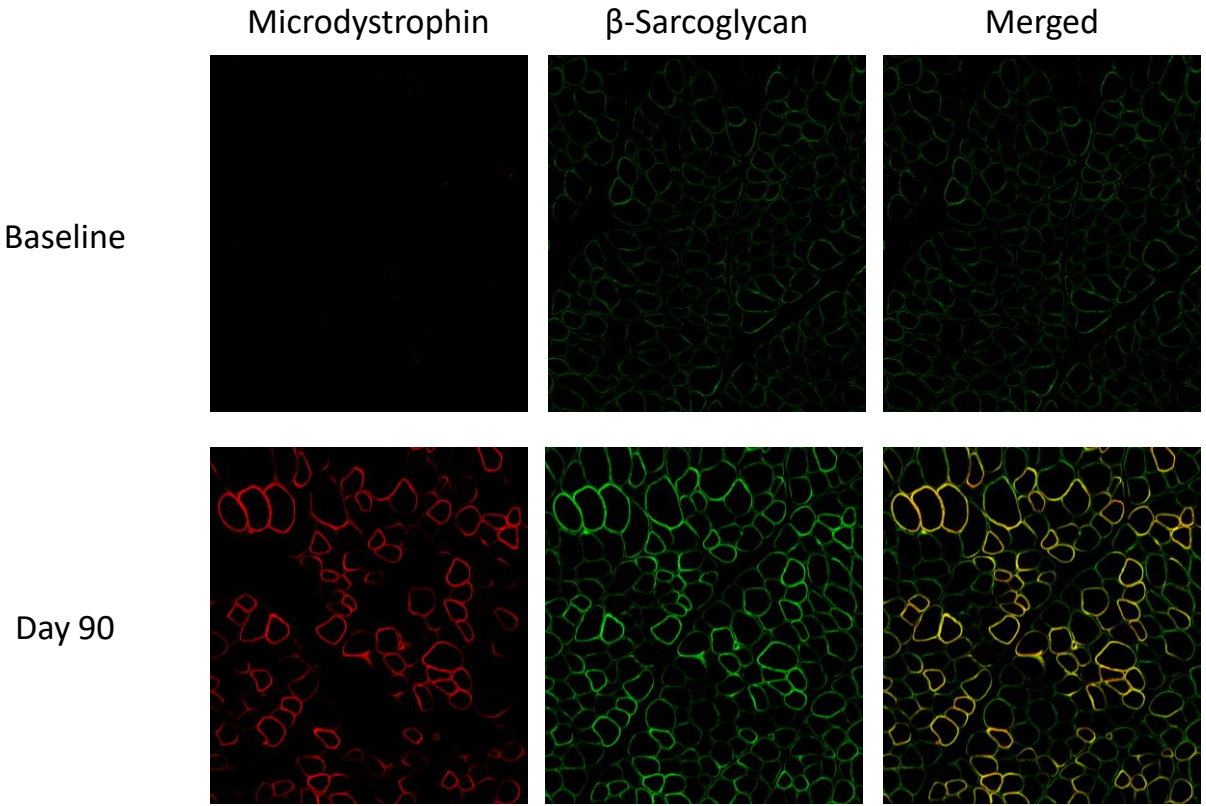
High magnification image confirms microdystrophin membrane localization





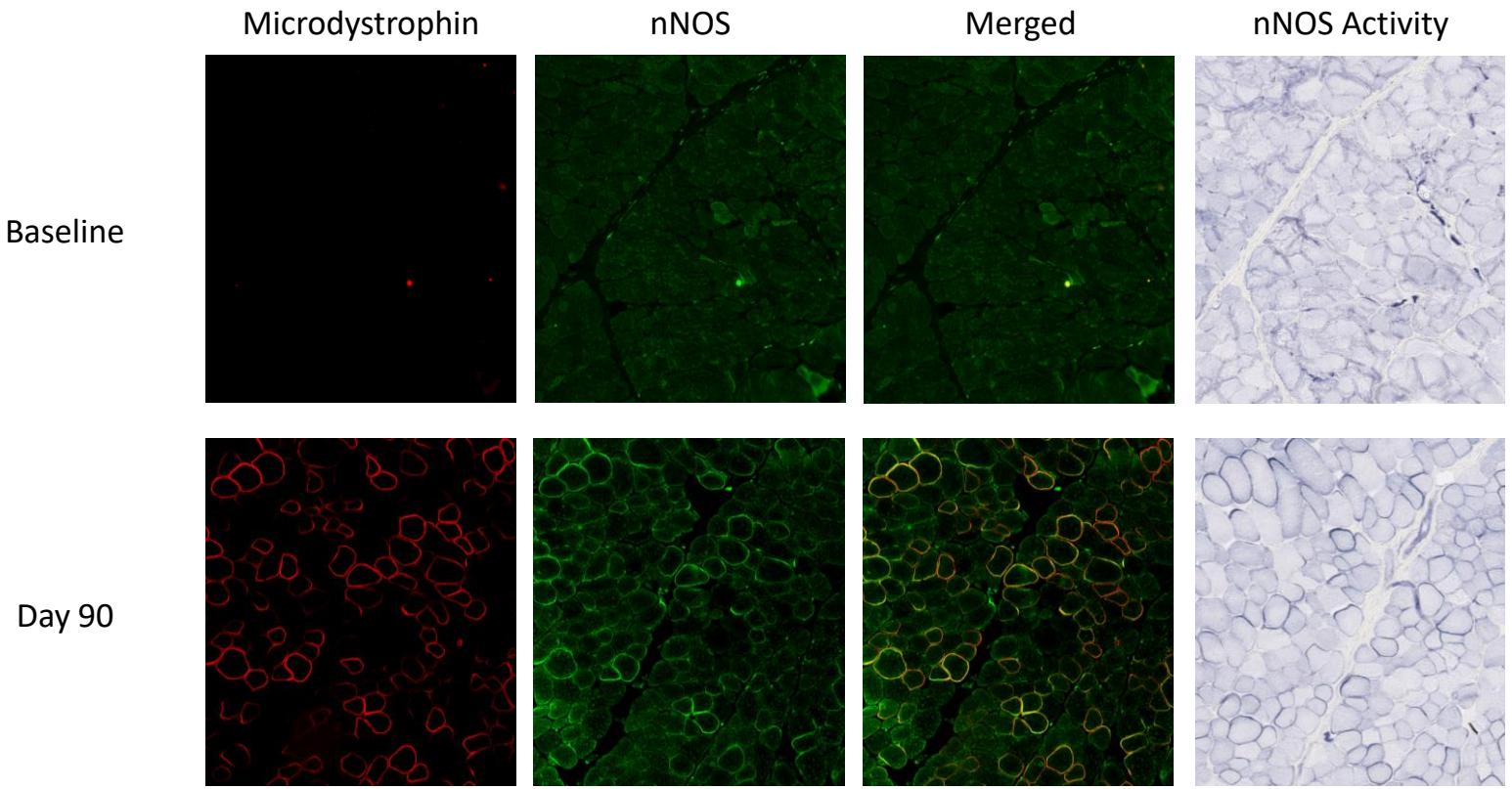
*Data presented at ASGCT 23rd Annual Meeting

Microdystrophin Expression in DMD Patients in 2E14 vg/kg Dose Cohort Resulted in Dystrophin Glycoprotein Complex Restoration



*Data presented at ASGCT 23rd Annual Meeting

Microdystrophin Expression in DMD Patients in 2E14 vg/kg Dose Cohort Further Resulted in Restored Enzymatically Active nNOS at the Sarcolemma

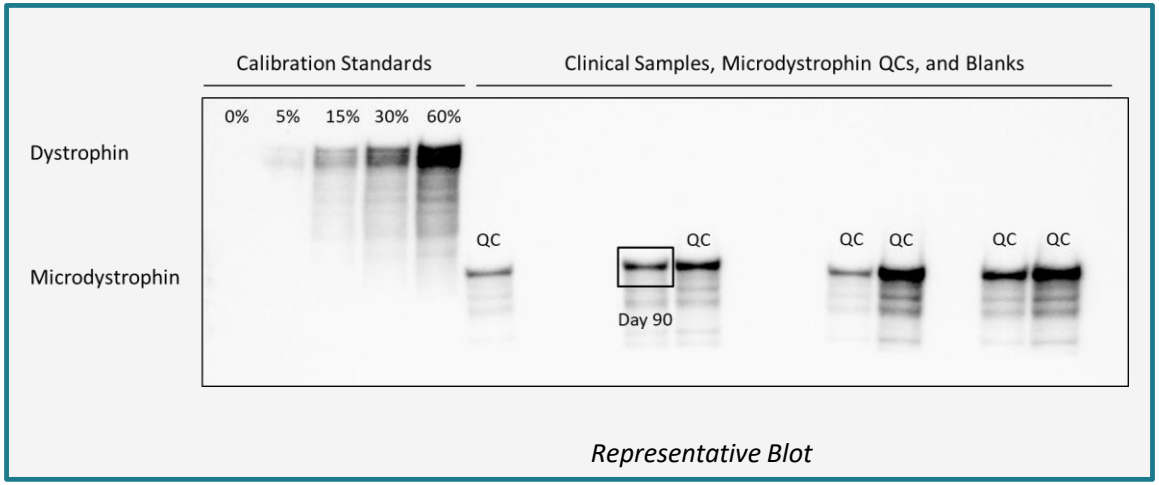


*Data presented at ASGCT 23rd Annual Meeting

Western Blot Quantification of Microdystrophin Protein Levels

Qualified western blot method

- 5%-60% of normal dystrophin quantifiable range
  - Pooled dystrophin positive and dystrophin negative human muscle protein extracts
- Microdystrophin QCs
  - Replicate samples at three levels (low, mid, high QCs)



Representative Blot

*Data presented at ASGCT 23rd Annual Meeting

SGT-001 Clinical Biomarker Results Support Continued Development

Three-month biopsies reported from patients 4, 5 and 6 dosed in the 2E14 vg/kg IGNITE DMD cohort

Immunofluorescence assays:

- 10%-20% of microdystrophin positive muscle fibers in 4th patient
- 50%-70% microdystrophin positive fibers in both the 5th and 6th patients
- Clear stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin in all patients

Western blot:

- Expression levels for 4th patient were detectable and estimated to be near the assay's level of quantification*
- Expression was 17.5% of normal control samples in 5th patient
- Expression was 8% of normal control samples in 6th patient

*5% of non-dystrophic control samples

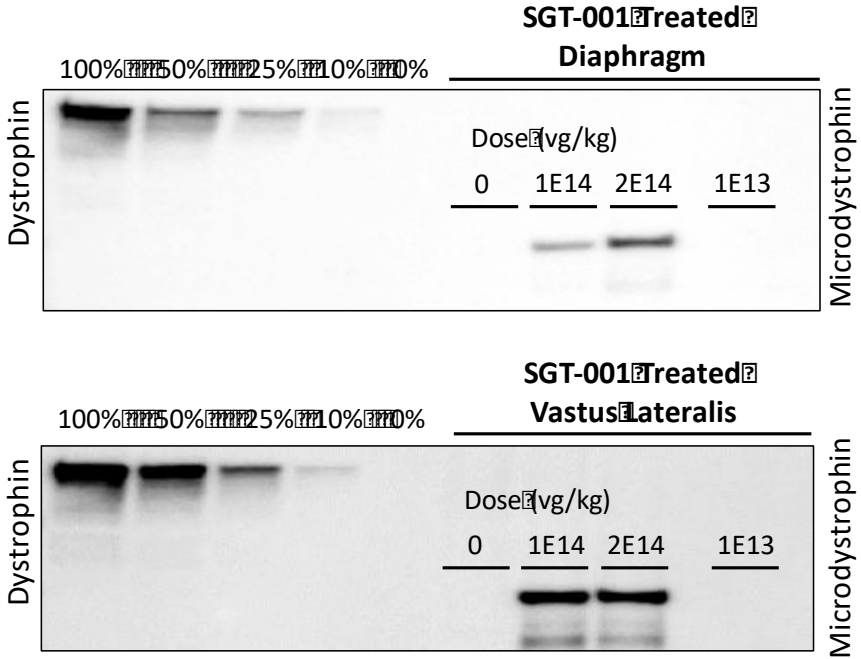
Expression of SGT-001 microdystrophin and nNOS function provide evidence that SGT-001 has the potential to provide meaningful therapeutic benefit for patients with Duchenne

SGT-001 Clinical Program

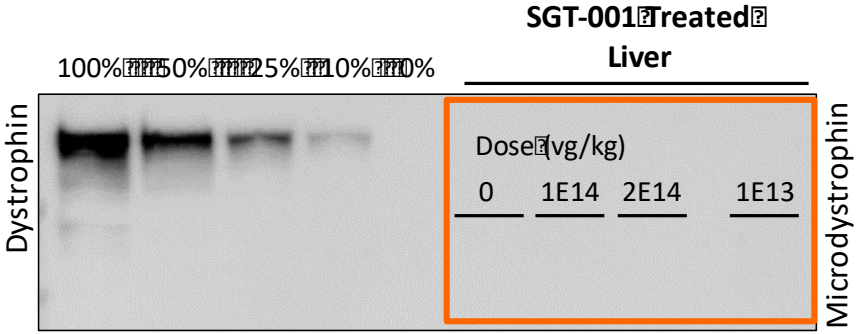
Select Preclinical Data

CK8 Muscle-Specific Promoter Restricted Expression To Muscles In Preclinical Studies

Target Tissue

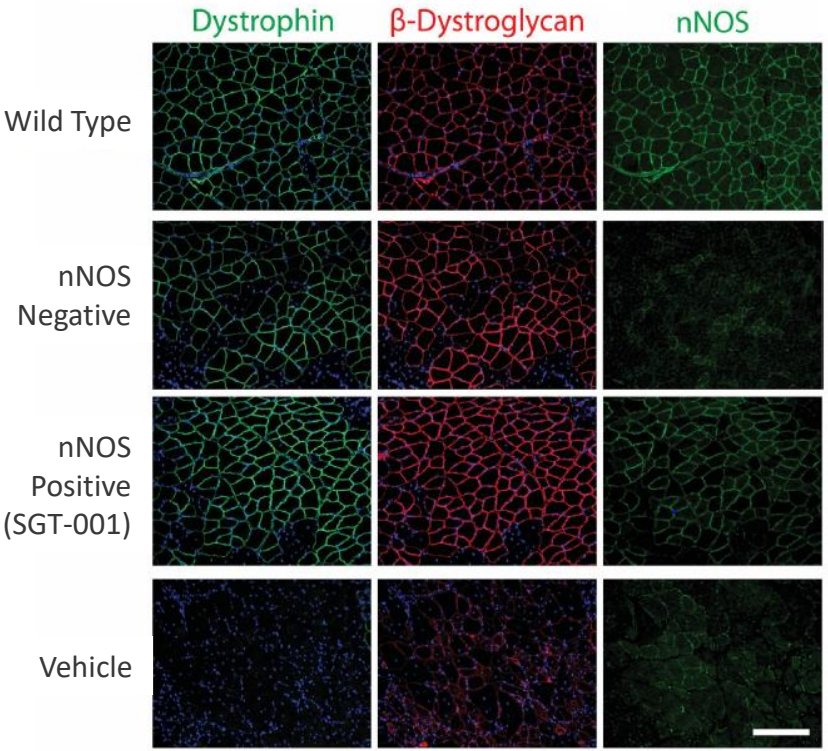


Non-target Tissue



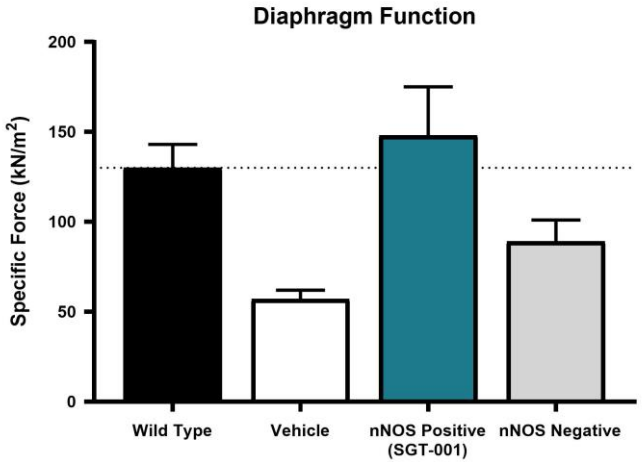
Company data. Three month efficacy study in GRMD canines. Representative only.

SGT-001 Microdystrophin With nNOS Binding Domain Selected Based On Extensive Comparative Analysis



Gastrocnemius cryosections from mdx mice.

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



Potential for nNOS related SGT-001 microdystrophin activity: Diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle

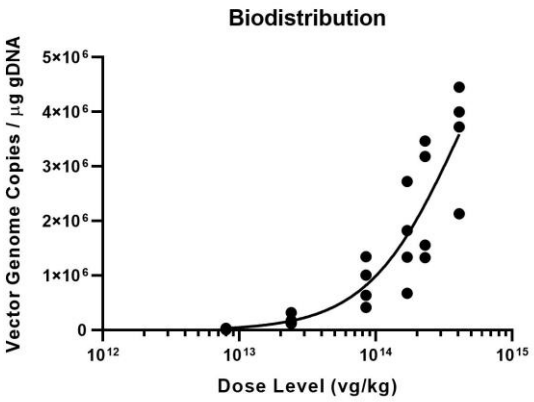
Ramos et al. Molecular Therapy, 2019.

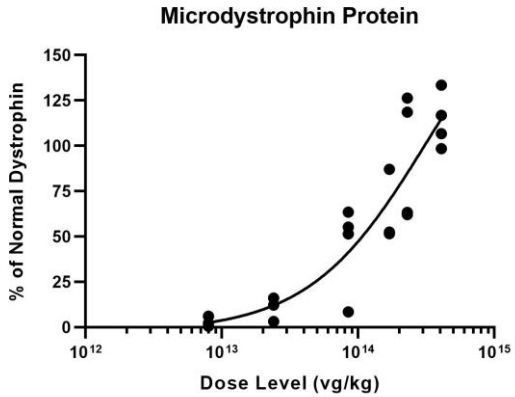
Dose Response In Preclinical Studies

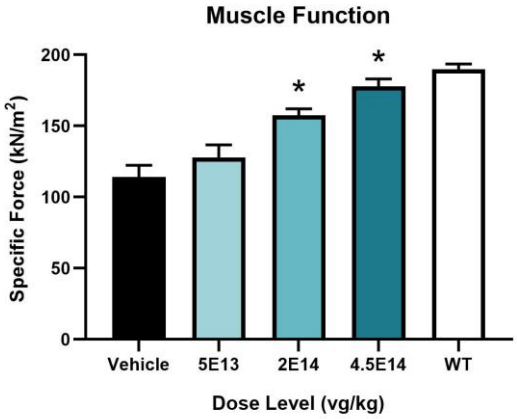
Exposure
How much vector gets into the tissue?

Target Engagement
How much protein is produced?

Functional Benefit

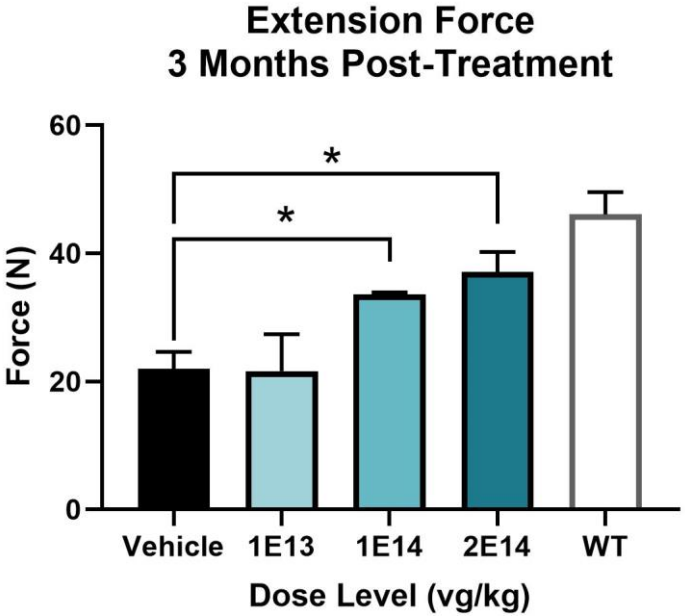


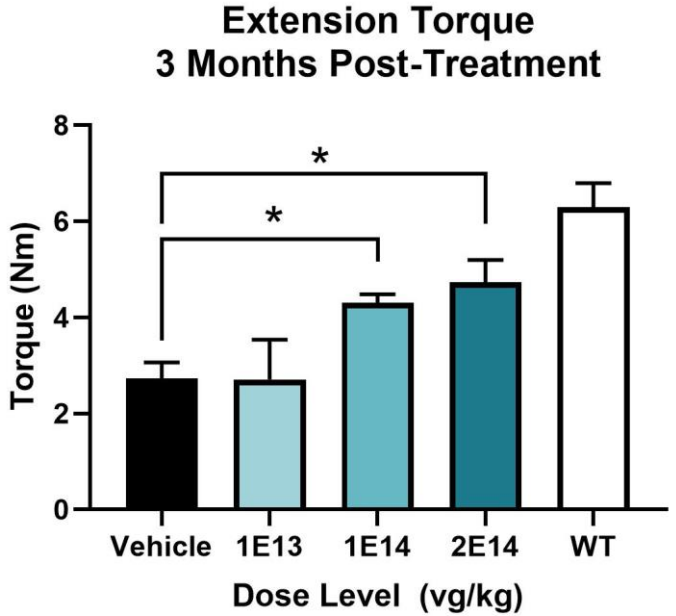




*Company data. mdx mouse studies | * p<0.05 statistical significance.*

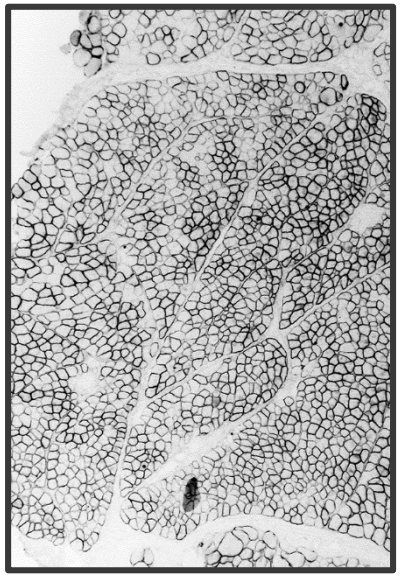
Significant Functional Benefit Demonstrated In Dystrophic Canines



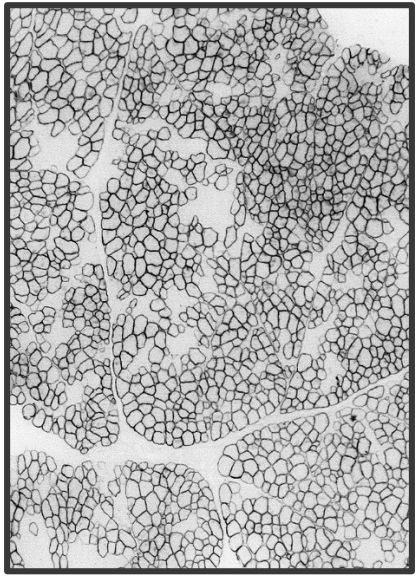


*Company data. Three month efficacy study in GRMD canines. n=3 per group | * p<0.05 statistical significance*

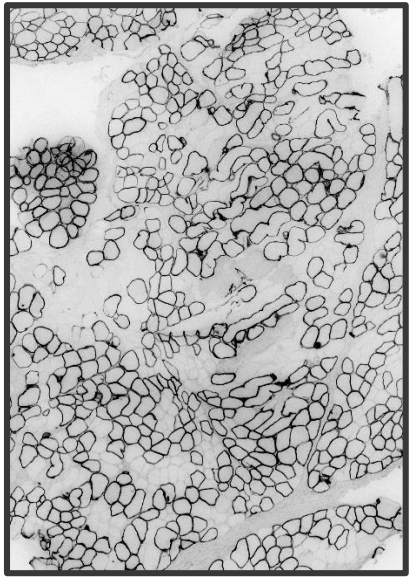
Long-term Durability Observed In Canines



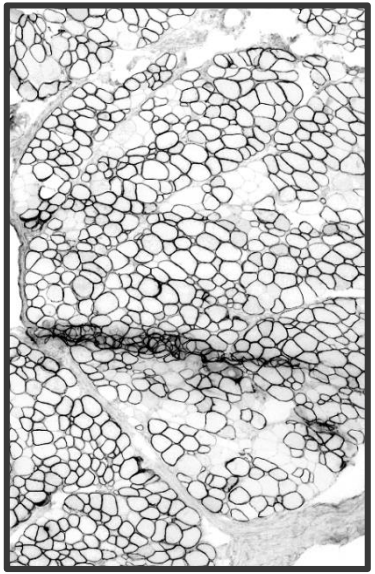
1 Month



3 Months



24 Months



30 Months

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

Manufacturing

Supplying the Market

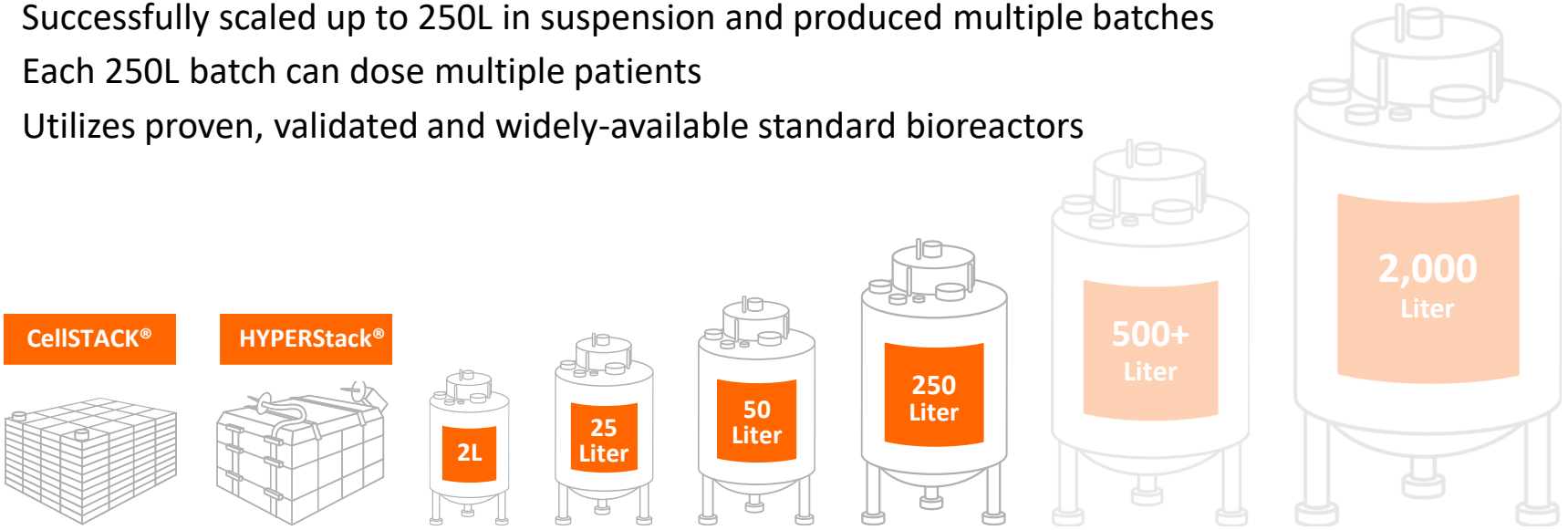
Addressing The DMD Gene Therapy Supply Challenge

Solid Manufacturing Strategy

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

GMP Manufacturing Process Has the Ability to Produce 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



Successful scale up to 250L suspension complete → Commercial scale → Further scale if needed

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Thank You