Solid Biosciences

Corporate Overview

October 2019

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, 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.

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Purpose-Built to Solve Duchenne Muscular Dystrophy (DMD)

360-Degree Approach

Differentiated Lead Gene Transfer

Scalable Manufacturing Process

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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.

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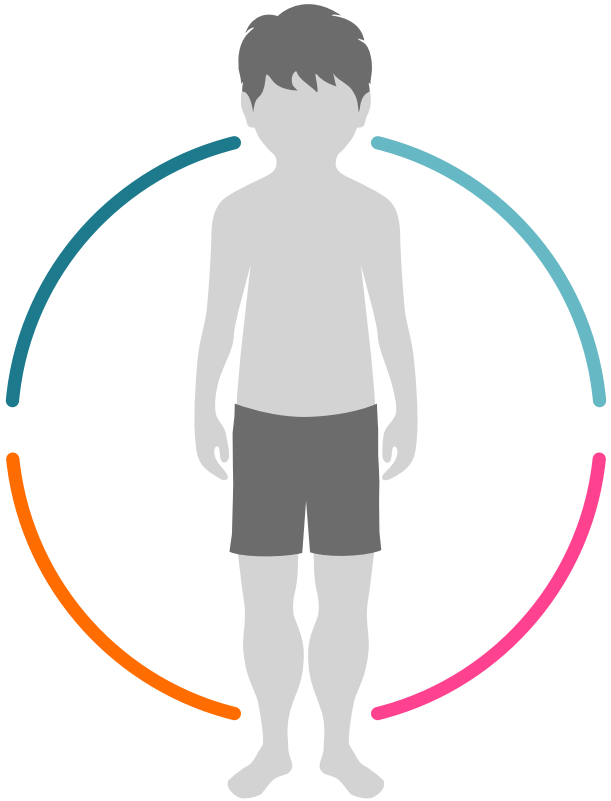
Solid Is Addressing The Full Spectrum Of Duchenne

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

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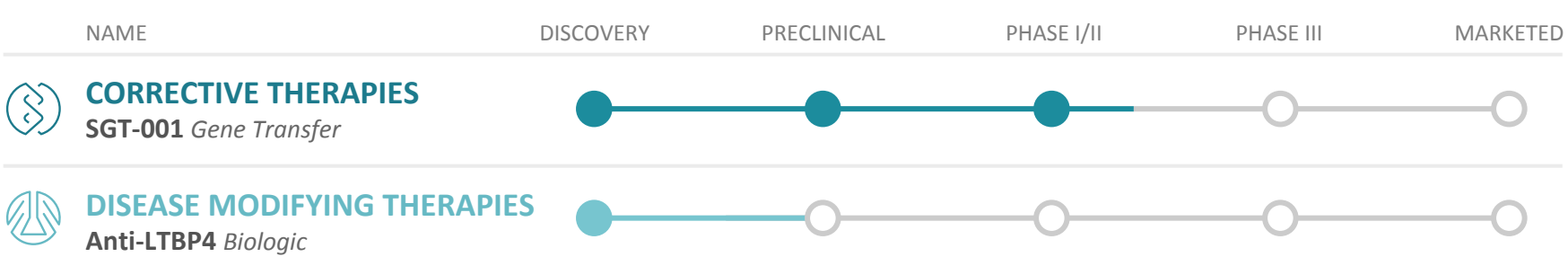
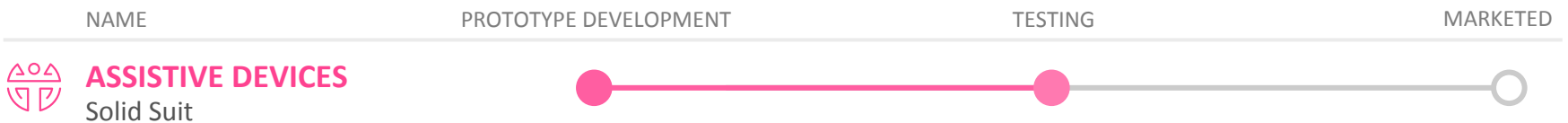
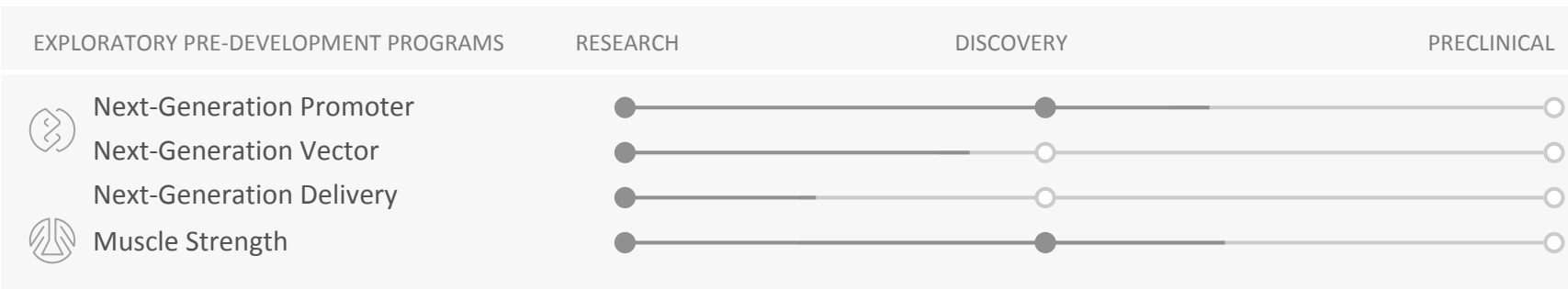
Solid Pipeline



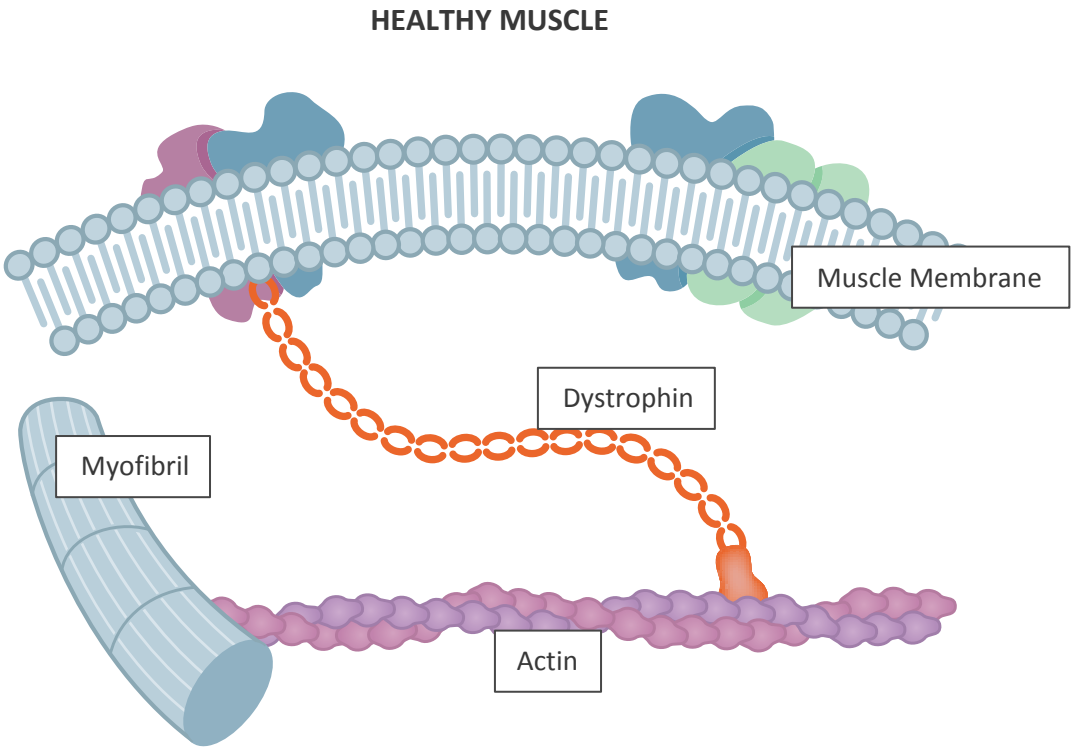




Corrective Therapies

Innovation in Gene Transfer

Gene Therapy To Address The Genetic Cause Of DMD



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

Visual representation only

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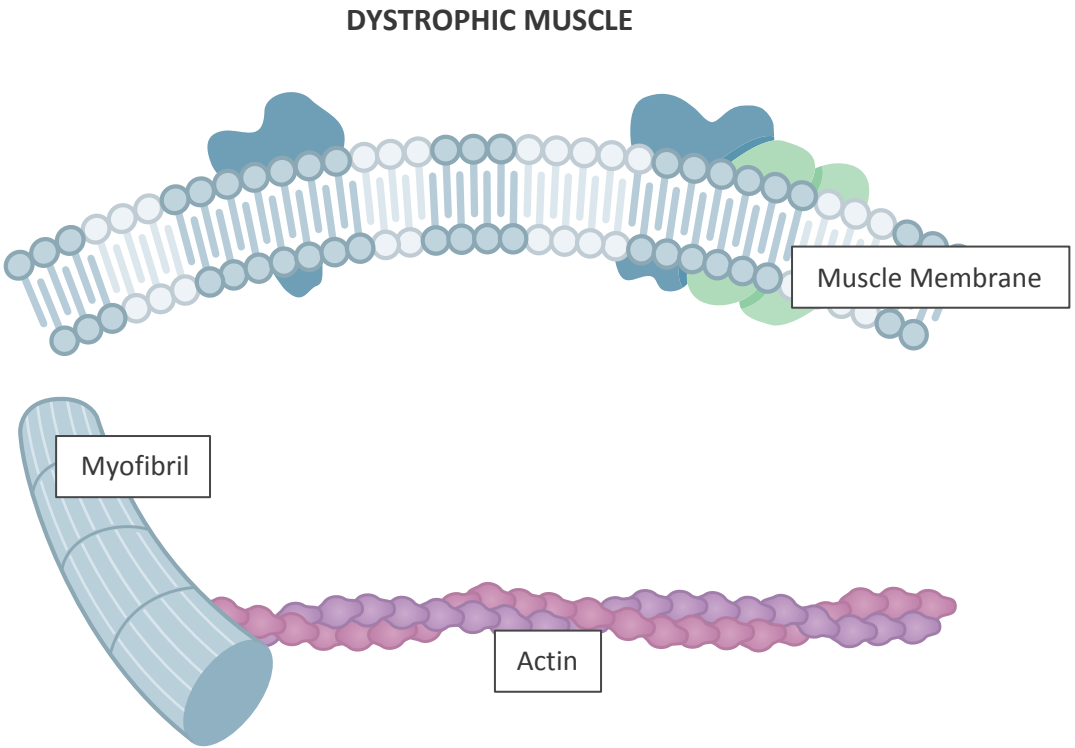
Gene Therapy To Address The Genetic Cause Of DMD



- 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

Visual representation only

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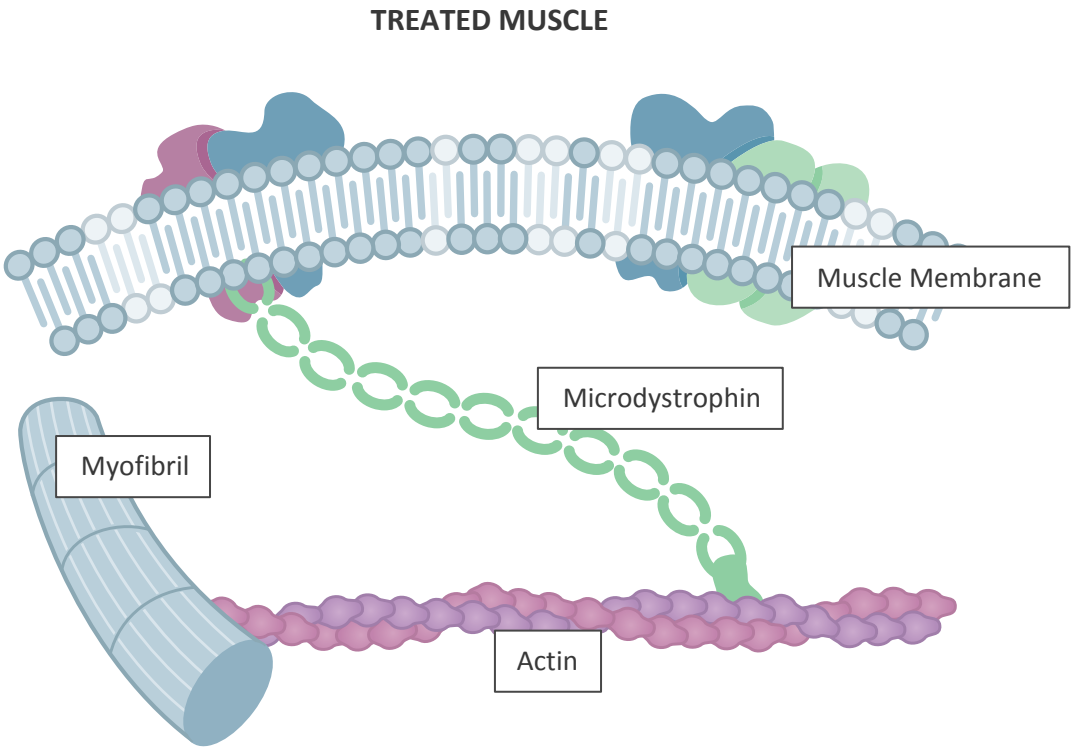
Gene Therapy To Address The Genetic Cause Of DMD



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

Visual representation only

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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**

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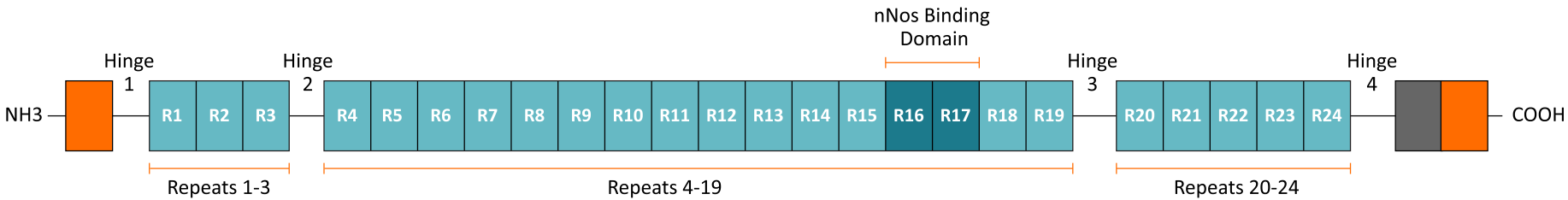
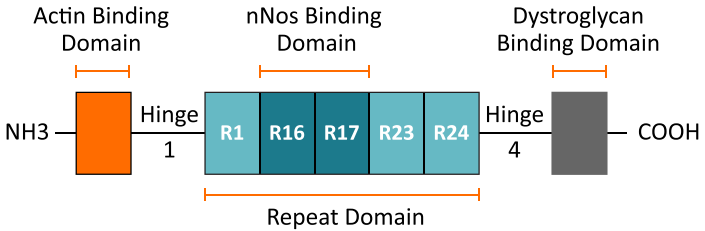
SGT-001 Microdystrophin Has A Differentiated Composition

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.

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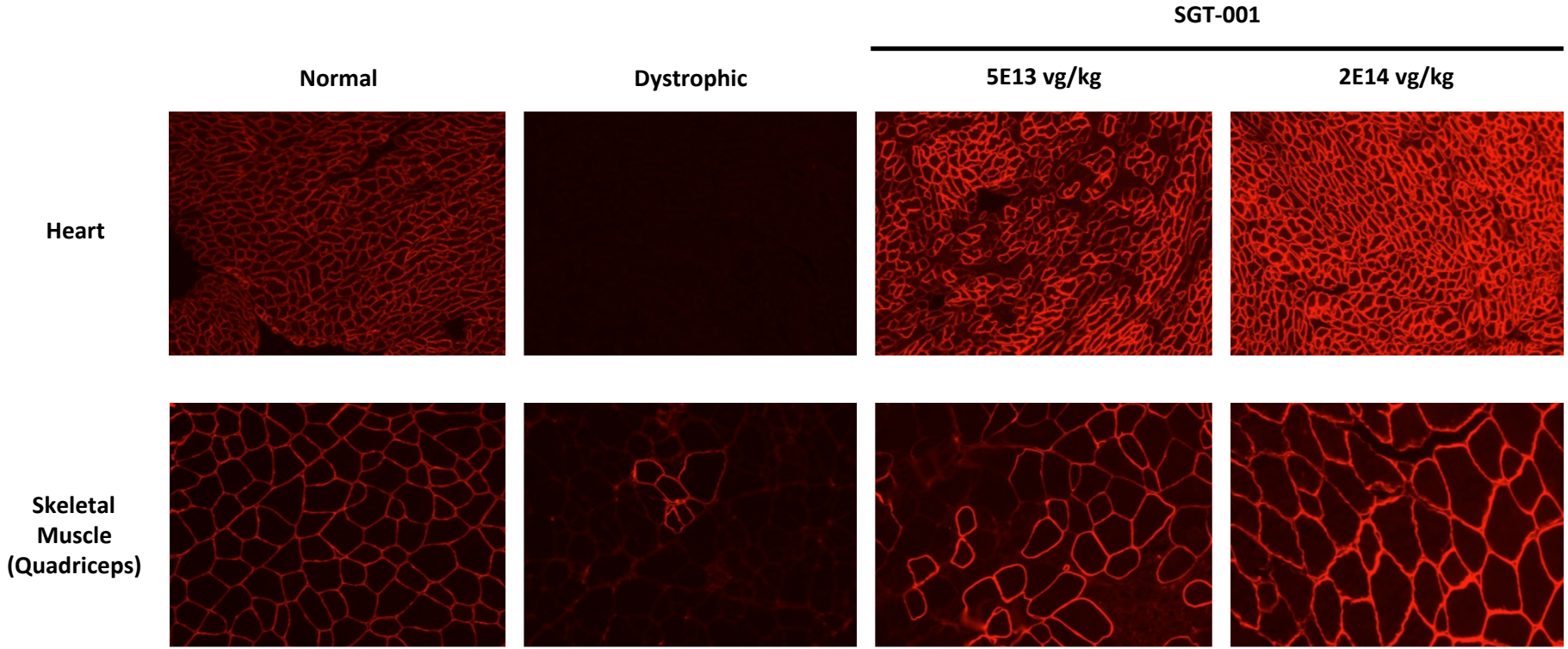
SGT-001 Promotes Significant Cardiac And Skeletal Muscle SGT-001 Microdystrophin Expression In Preclinical Models



Company data. Mouse studies.

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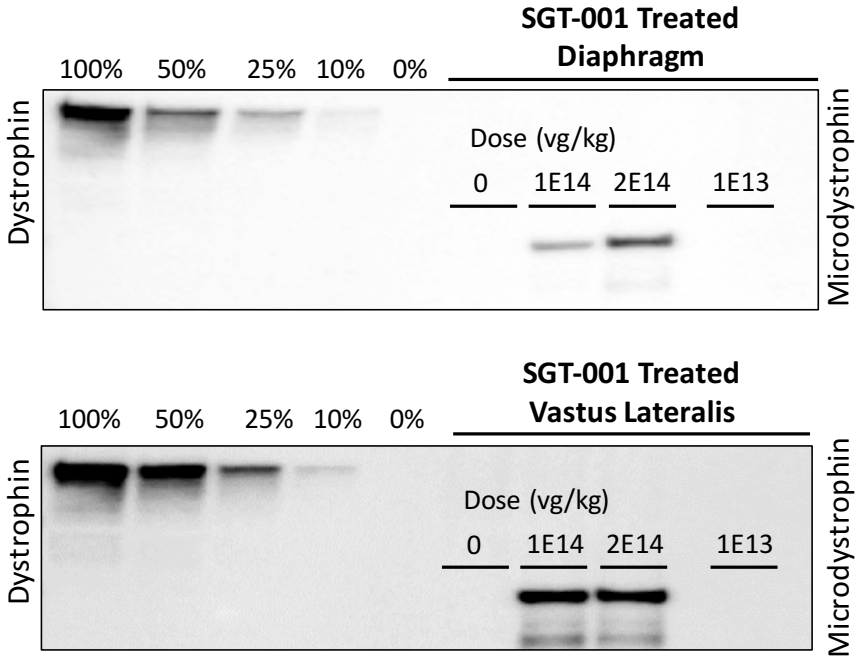
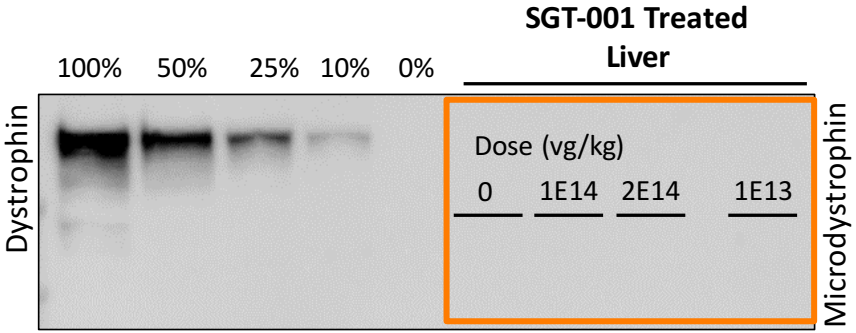
CK8 Muscle-Specific Promoter Restricts Expression To Muscles In Preclinical Studies

Target Tissue



Non-target Tissue



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

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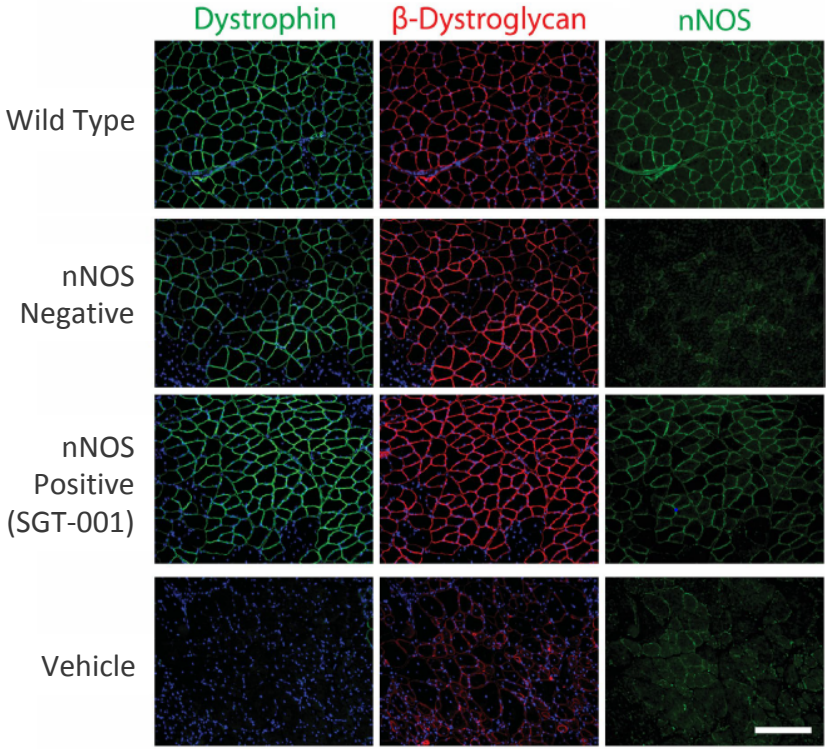
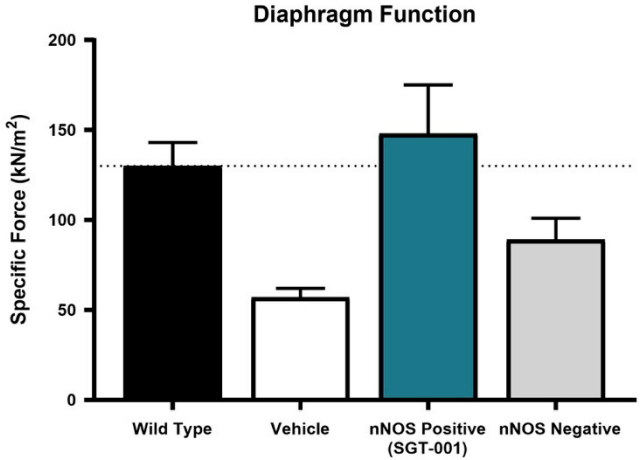
SGT-001 Microdystrophin With nNOS Binding Domain Selected Based On Extensive Comparative Analysis



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.

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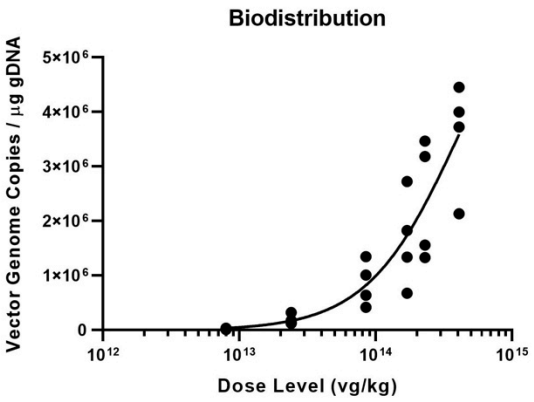
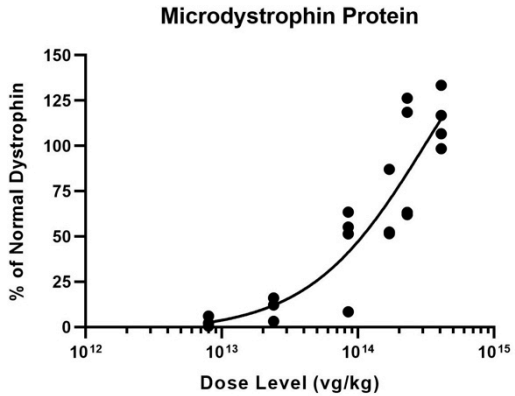
Dose Response In Preclinical Studies

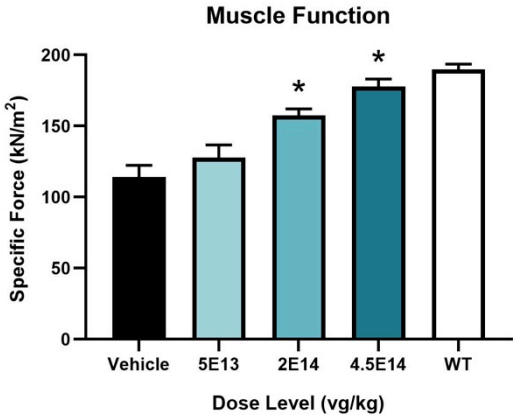
Exposure
How much vector gets into the tissue?

Target Engagement
How much protein is produced?

Functional Benefit







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

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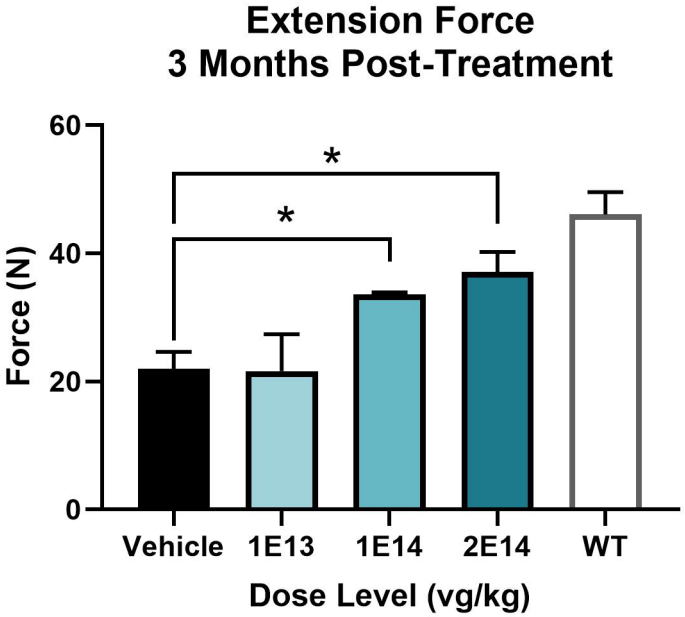
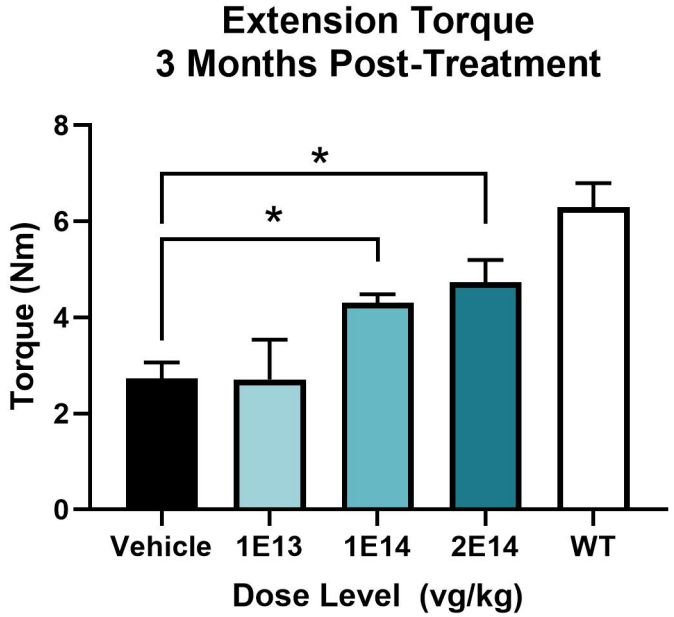
Significant Functional Benefit Demonstrated In Dystrophic Canines





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

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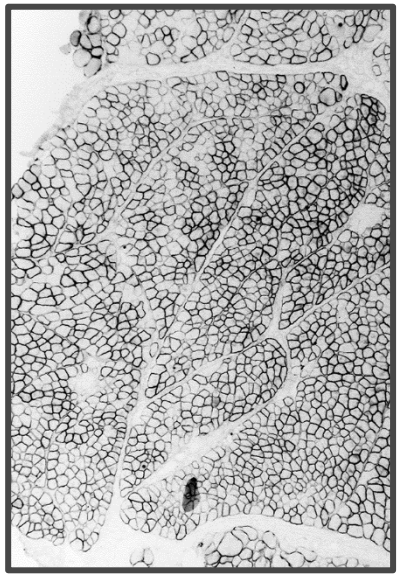
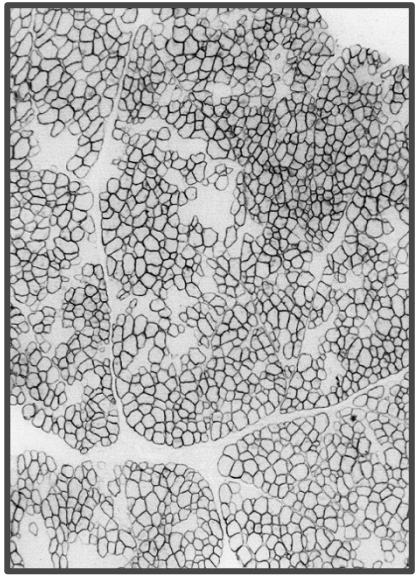
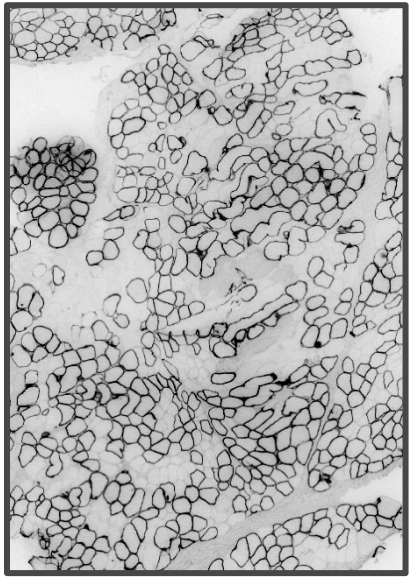
Long-term Durability Observed In Canines



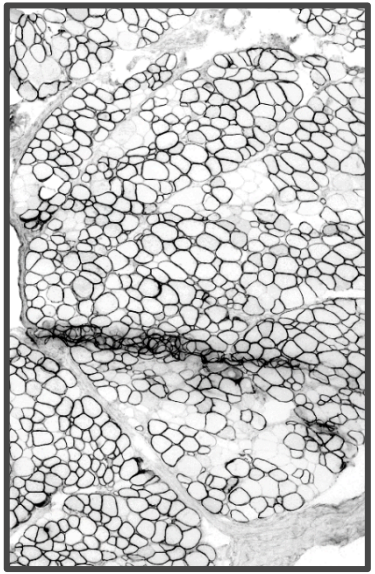
1 Month



3 Months



24 Months



30 Months

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

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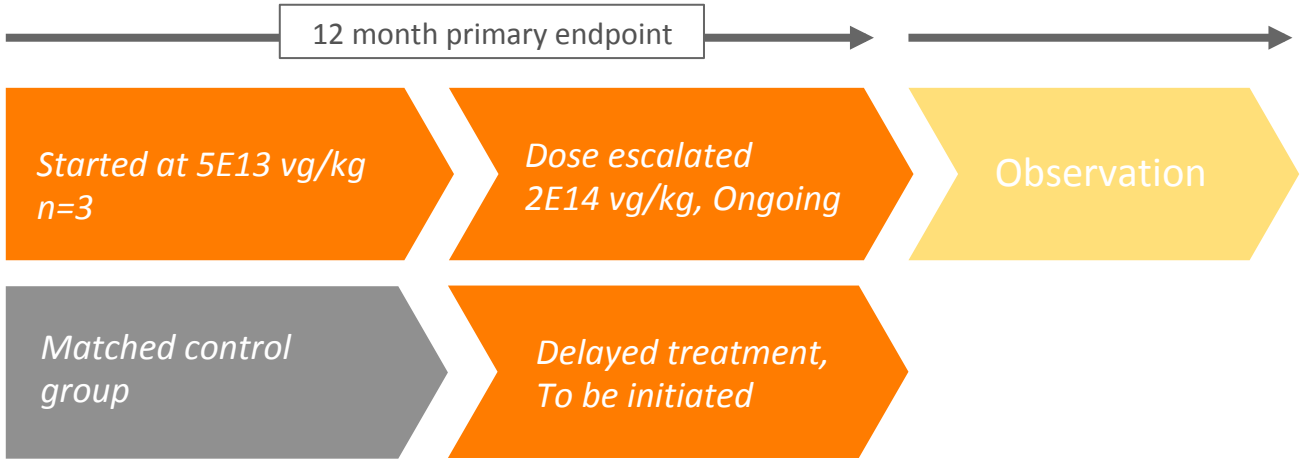
SGT-001 Clinical Program

IGNITE DMD

SGT-001 Phase I/II Clinical Study

IGNITEDMD



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)

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Manufacturing

Producing Materials

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

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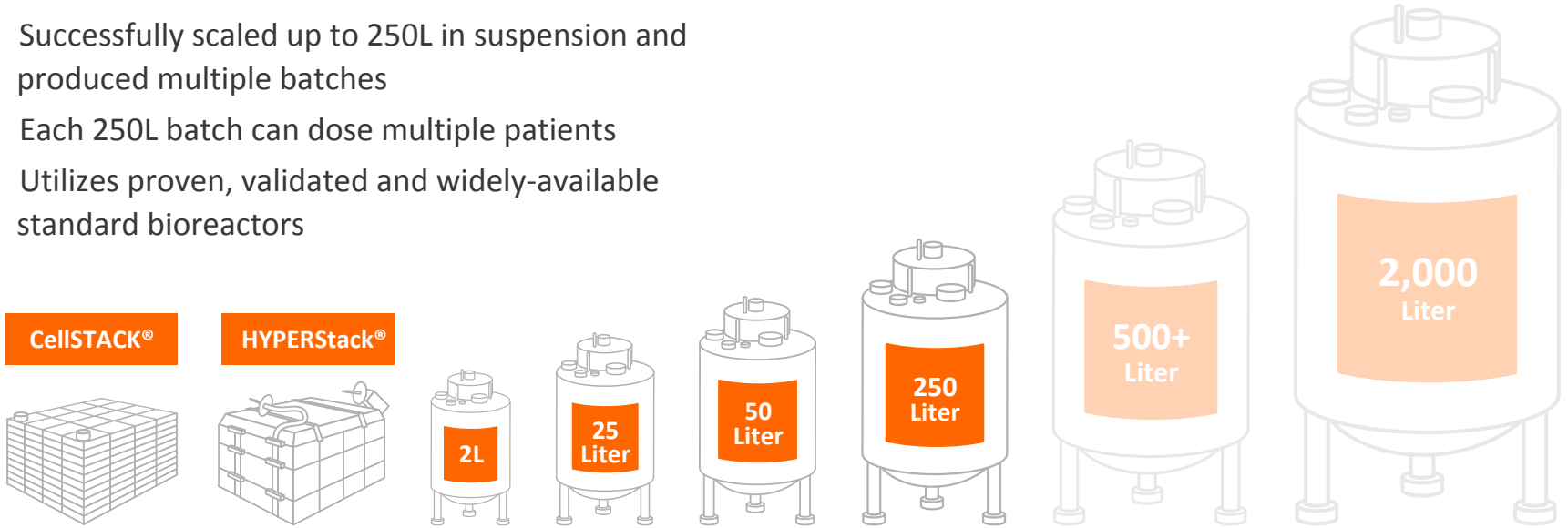
GMP Manufacturing Process Currently Producing At Significant Volume

- 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



Successful scale up to 250L suspension complete

Potential to scale up to further increase yield and manufacturing efficiency

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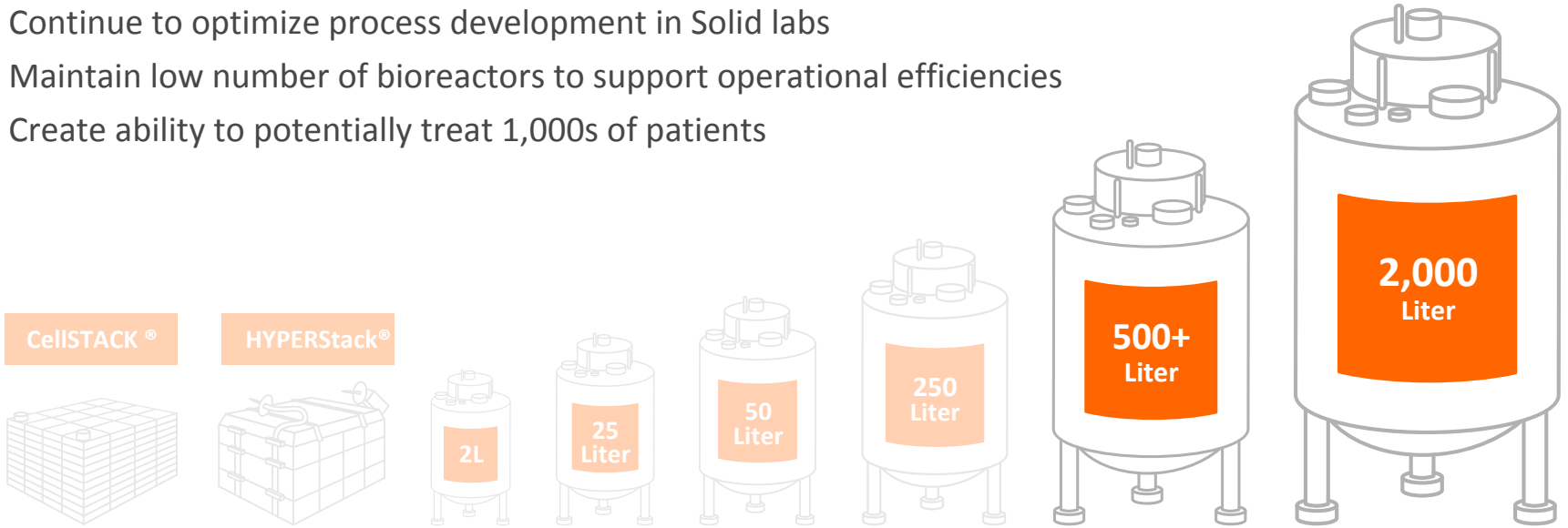
Scaling Process To Efficiently Supply Commercial Markets

- 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



Successful scale up to 250L complete

Potential to scale up to further increase yield and manufacturing efficiency

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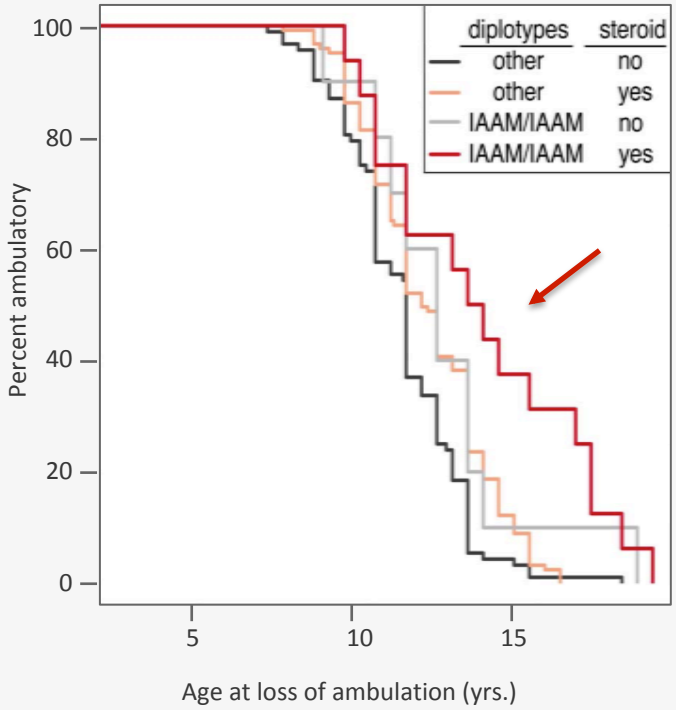
LTBP4 and Next Generation Gene Therapies

Expanding Pipeline

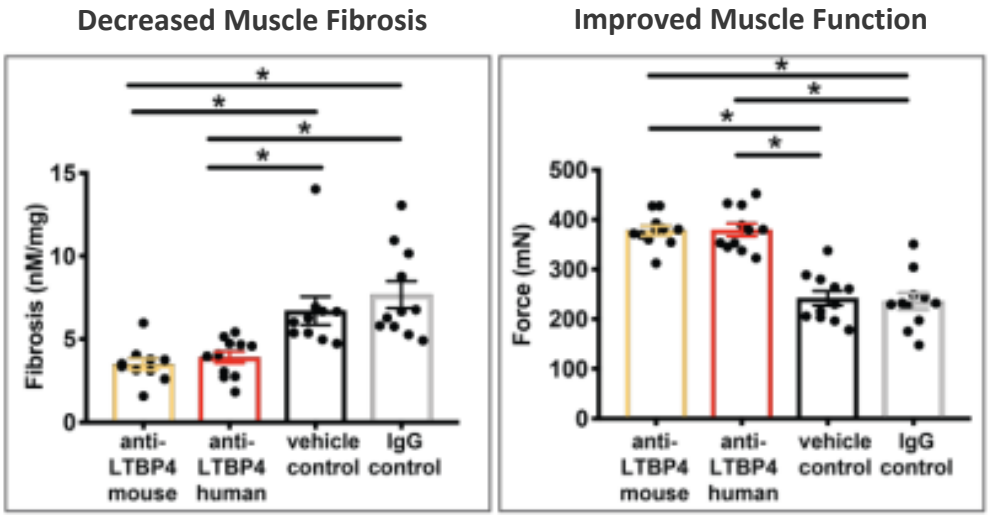
Anti-LTBP4 Disease-Modifying Therapy To Address Fibrosis

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 24 wks

Ikaika Therapeutics

Northwestern Medicine® Feinberg School of Medicine

Demonbreun, Quattrocelli, McNally – unpublished data.

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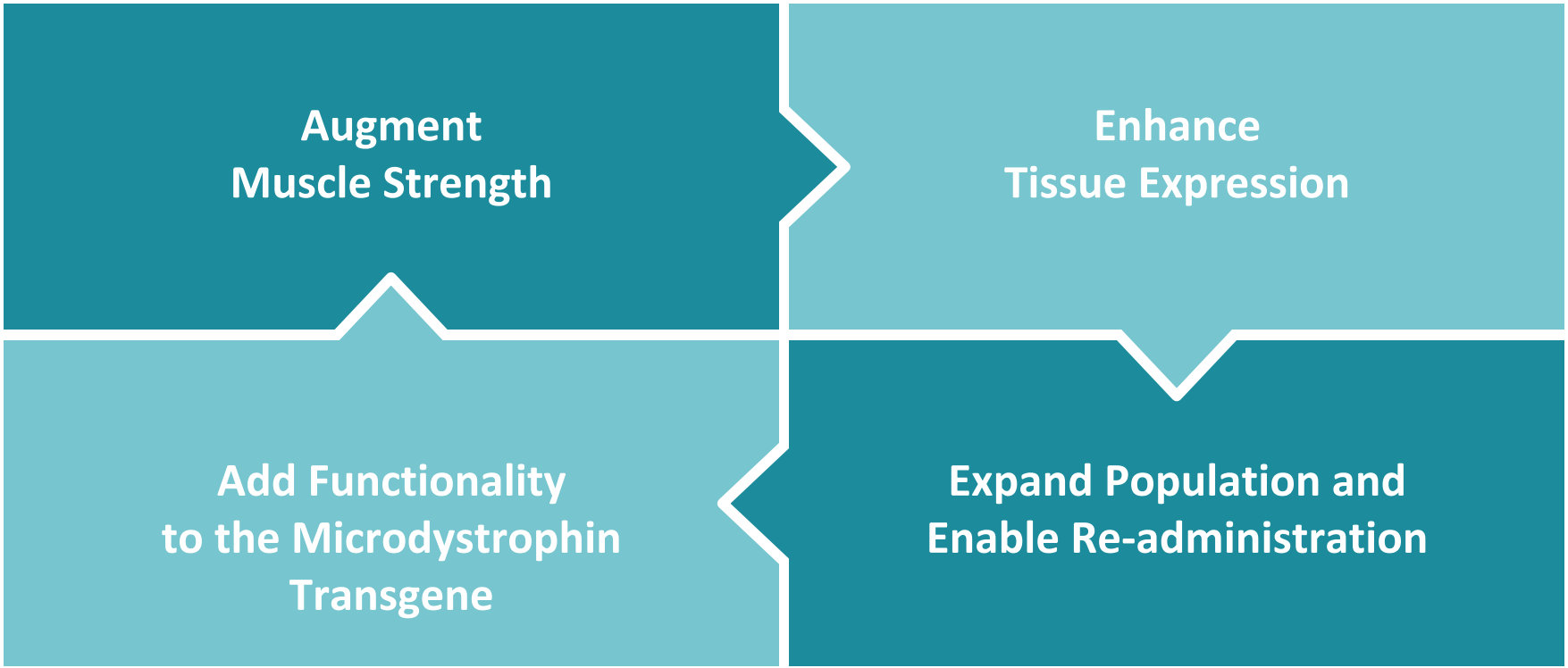
Internal And Partnered Programs To Build Comprehensive Pipeline For Duchenne



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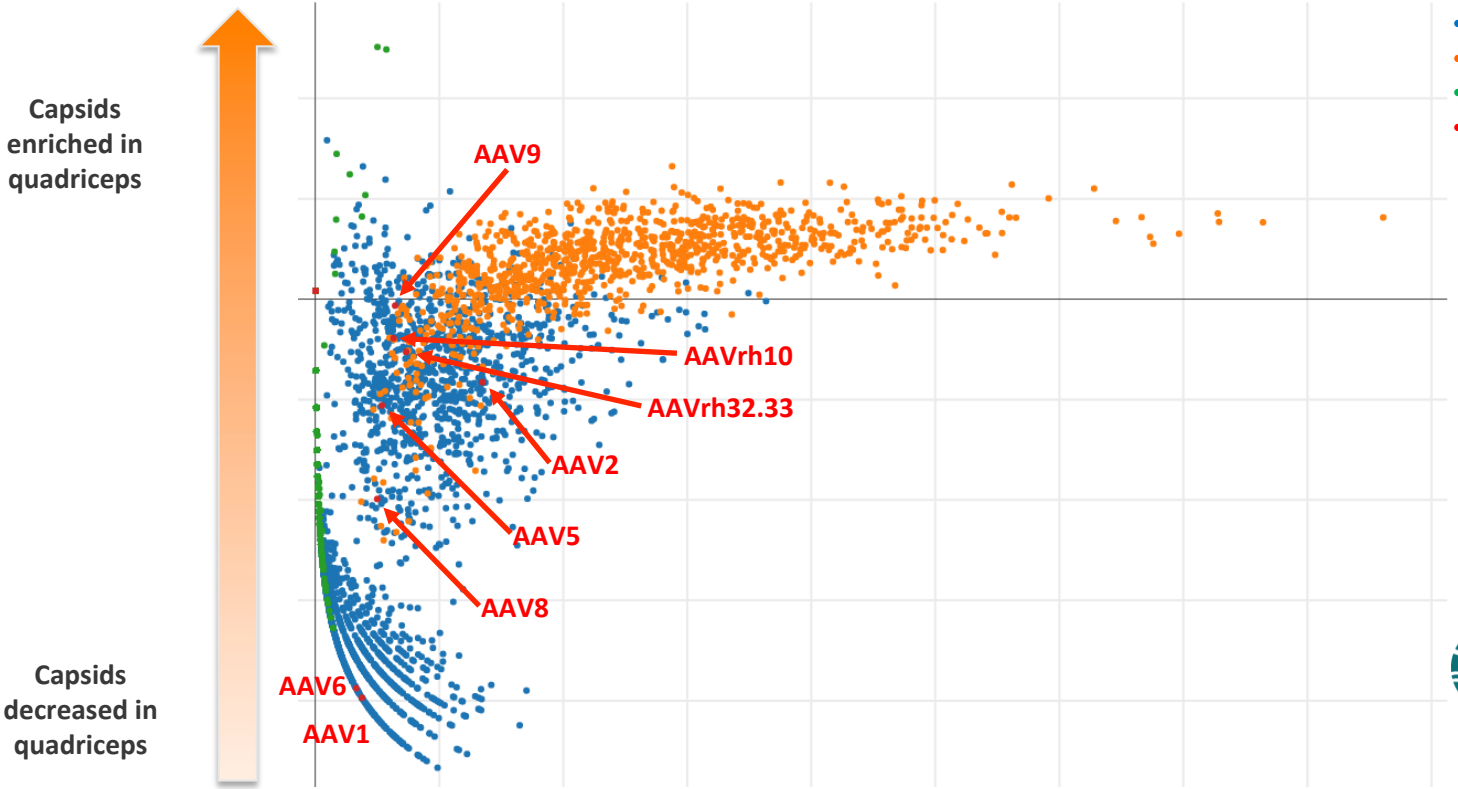
Next Generation Screening in Disease-Specific Models Yields Novel Potential AAV Candidates



Lonza

Massachusetts Eye and Ear®
sense life. experience life.

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Continued Progress

SGT-001 Clinical Data

- Data from second dose cohort later this year

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

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