Microdystrophin Gene Therapy – SGT-001

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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, 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

Scalable Manufacturing Process
Meet clinical and commercial needs

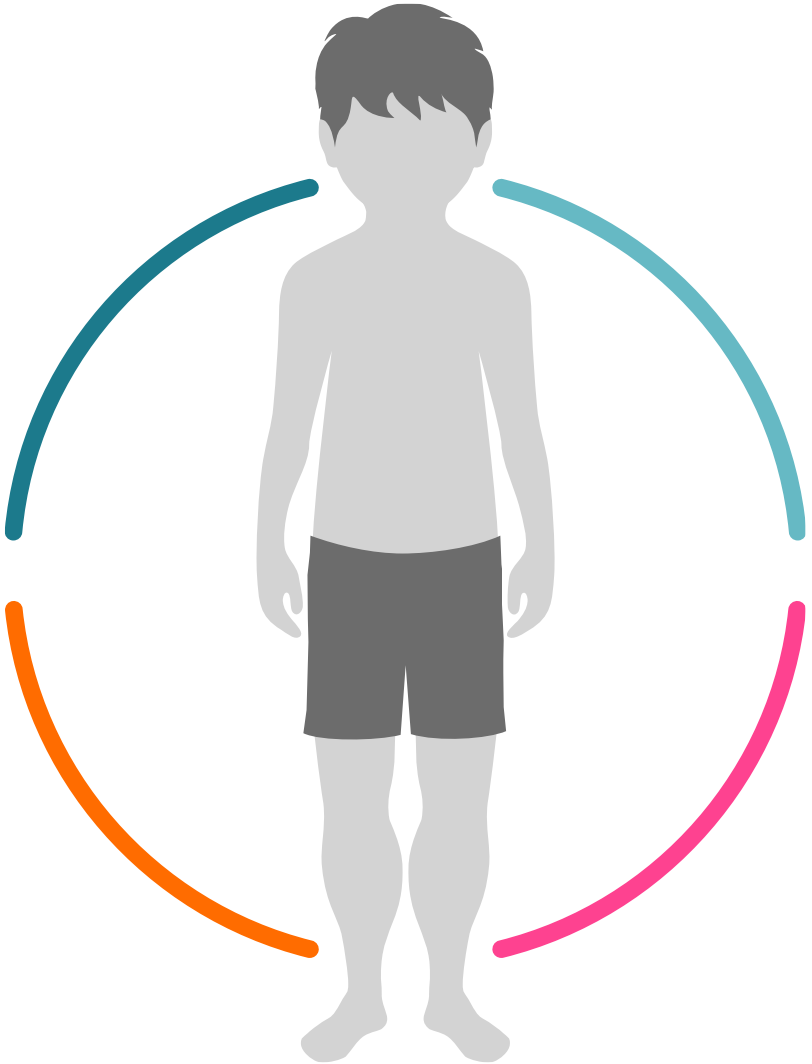
Solid Is Addressing The Full Spectrum Of Duchenne

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

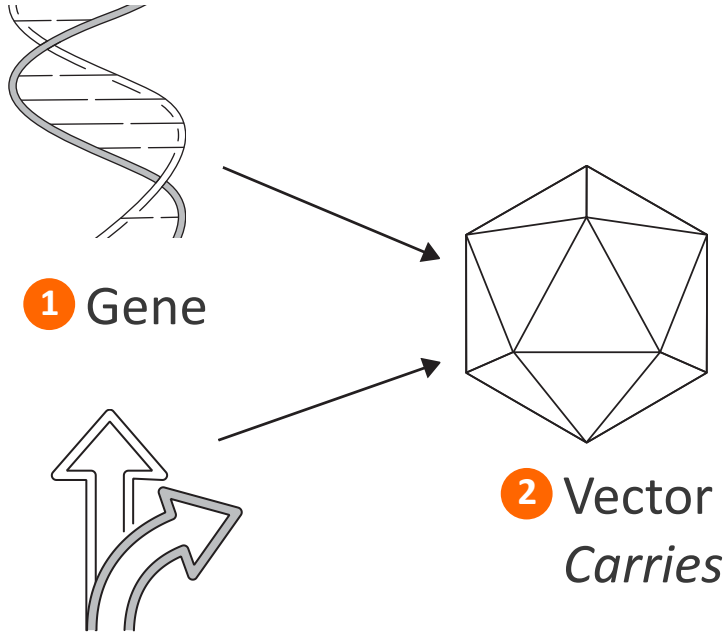
Technology to support mobility

Corrective Therapies

Gene Transfer – SGT-001

What Is Gene Transfer For DMD?

Gene transfer for DMD is made up of three essential elements:

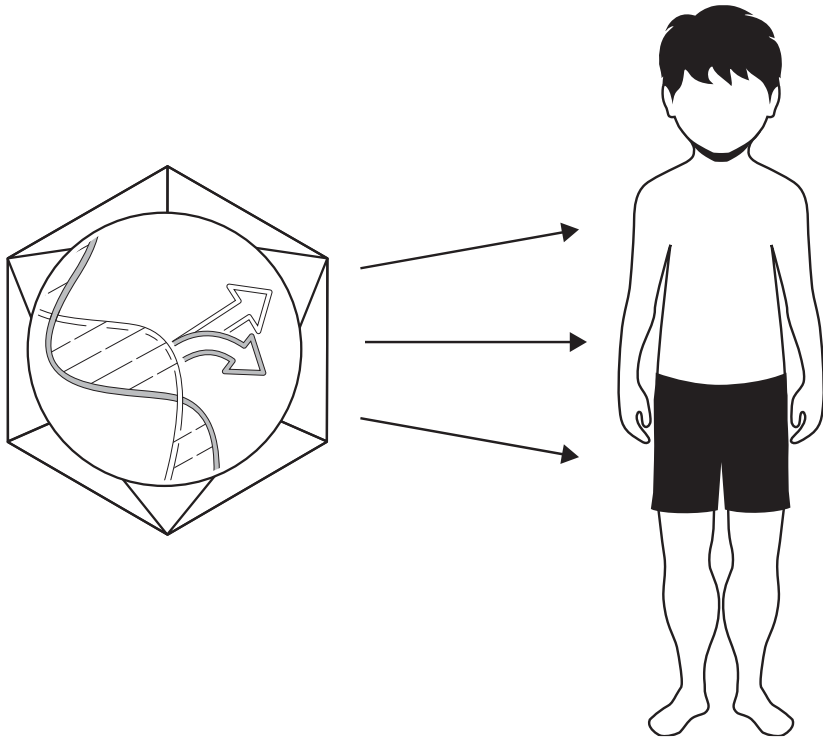


1 Gene

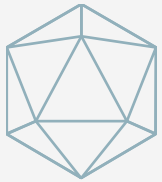
2 Vector
Carries the gene

2 Promoter
Controls expression

The combined product is then administered to the patient

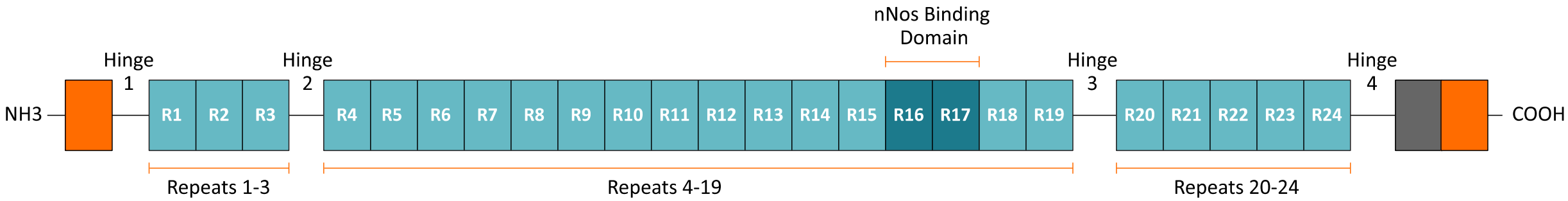


Each Component Of SGT-001 Was Carefully Selected

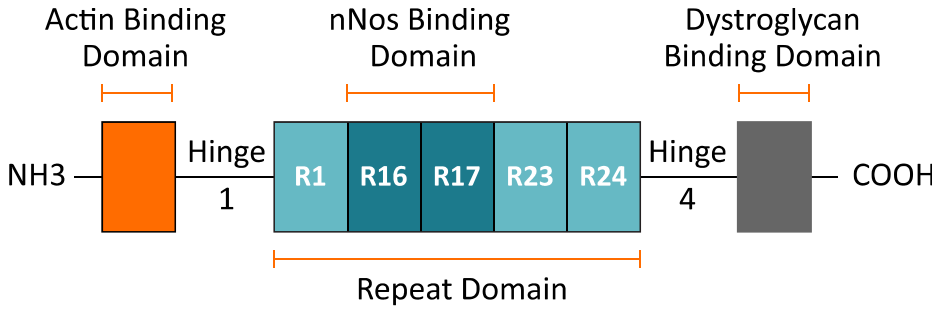
 **Transgene**	Restore key functions of a complex protein	**SGT-001 microdystrophin**
 **Promoter**	Expression is highly targeted	**CK8**
 **Vector**	Skeletal and cardiac transduction	**AAV9**

SGT-001 Microdystrophin Has A Differentiated Composition

Full Length Dystrophin Protein



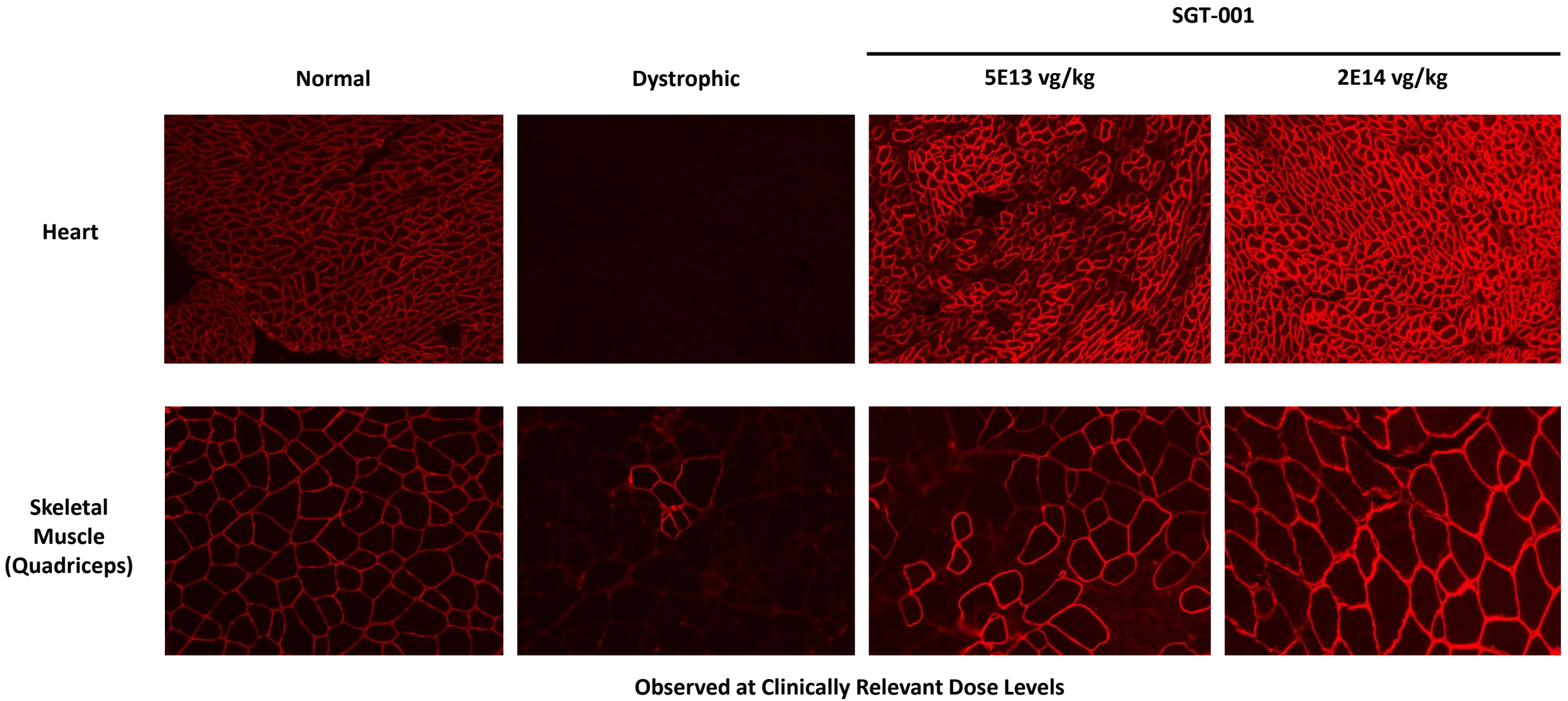
SGT-001 Microdystrophin Protein



- Microdystrophin construct DNA able to fit in AAV9 vector
- Contains 5 structural domains and uniquely includes the nNOS binding domain
- SGT-001 selection based on more than 30 years of research; confirmed through internal comparative analysis

Visual representation only

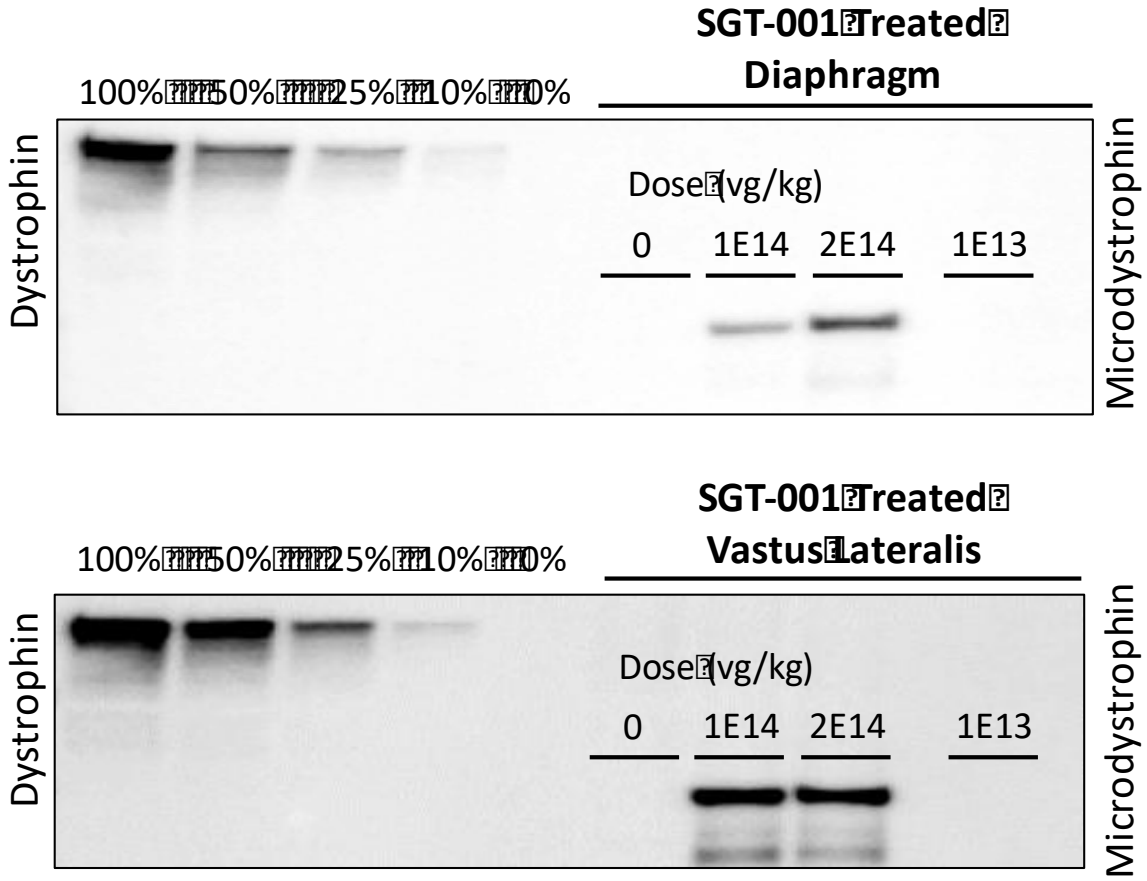
SGT-001 Promotes Significant Cardiac And Skeletal Muscle Microdystrophin Expression in dystrophic (mdx) mice



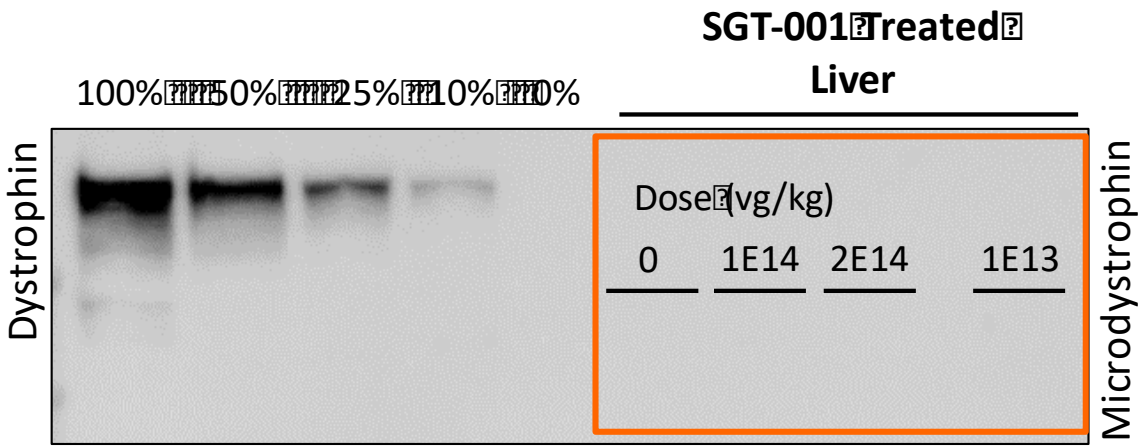
Observed at Clinically Relevant Dose Levels

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

Target Tissue



Non-target Tissue



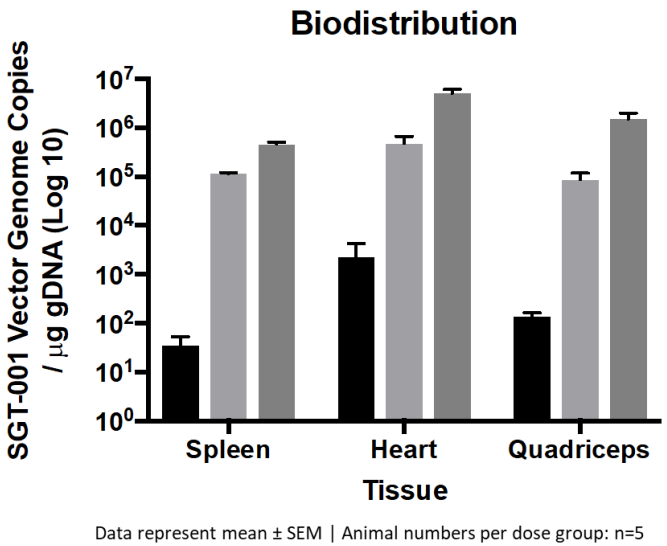
Samples analyzed 90 days post administration. Representative only.

Dose-escalation - Linking Dose Level To Function In Preclinical Studies

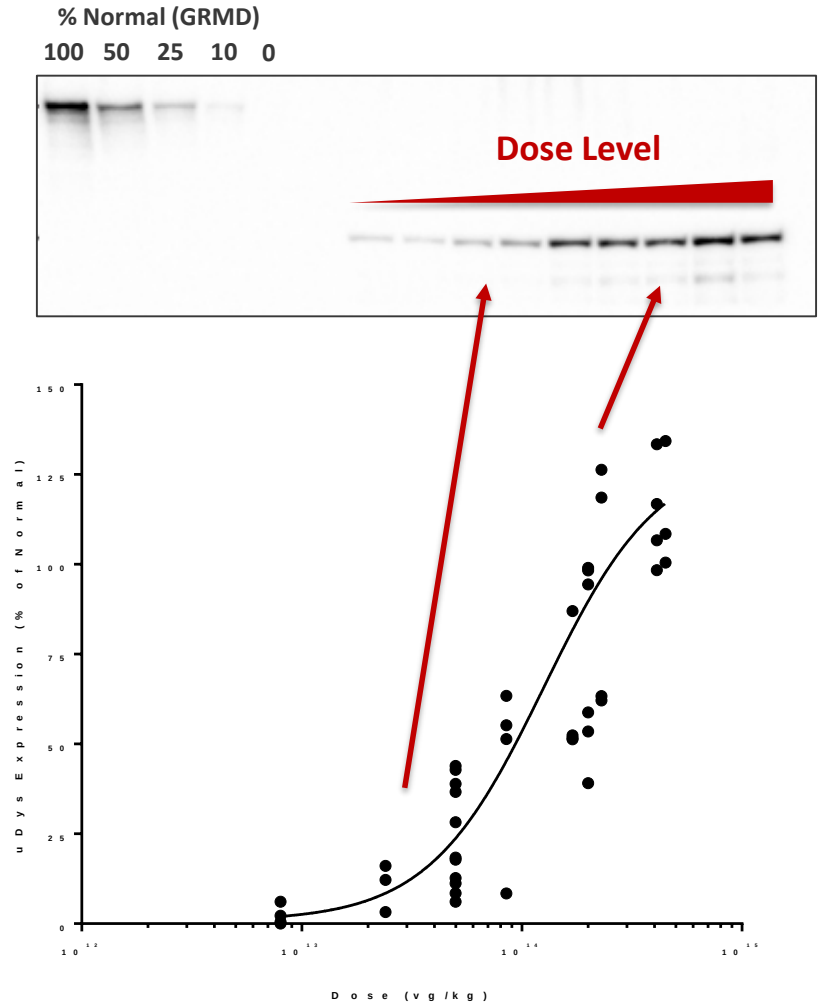
Exposure
(How much vector (drug) gets into the tissue?)

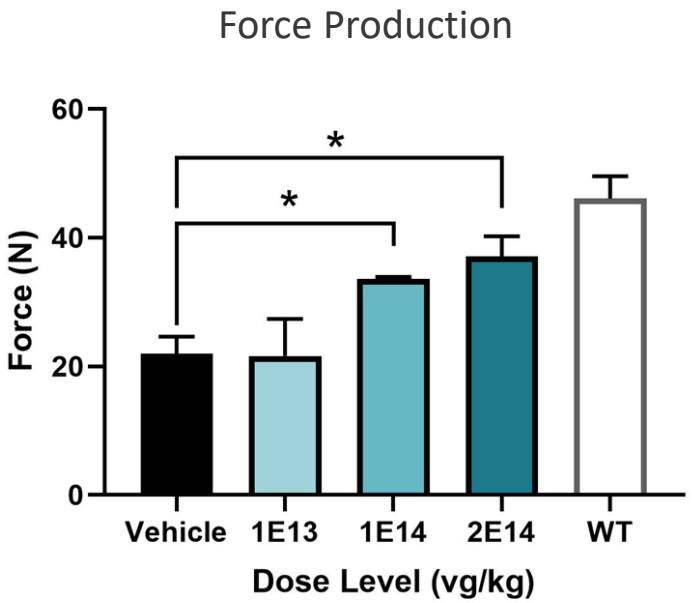
Target Engagement
(How much protein is produced?)

Efficacy / Benefit
(What functional changes are observed?)



Data represent mean ± SEM | Animal numbers per dose group: n=5





(3-month post dose data from GRMD study)

SGT-001 Clinical Program

IGNITE DMD

SGT-001 Phase I/II Clinical Study Ongoing

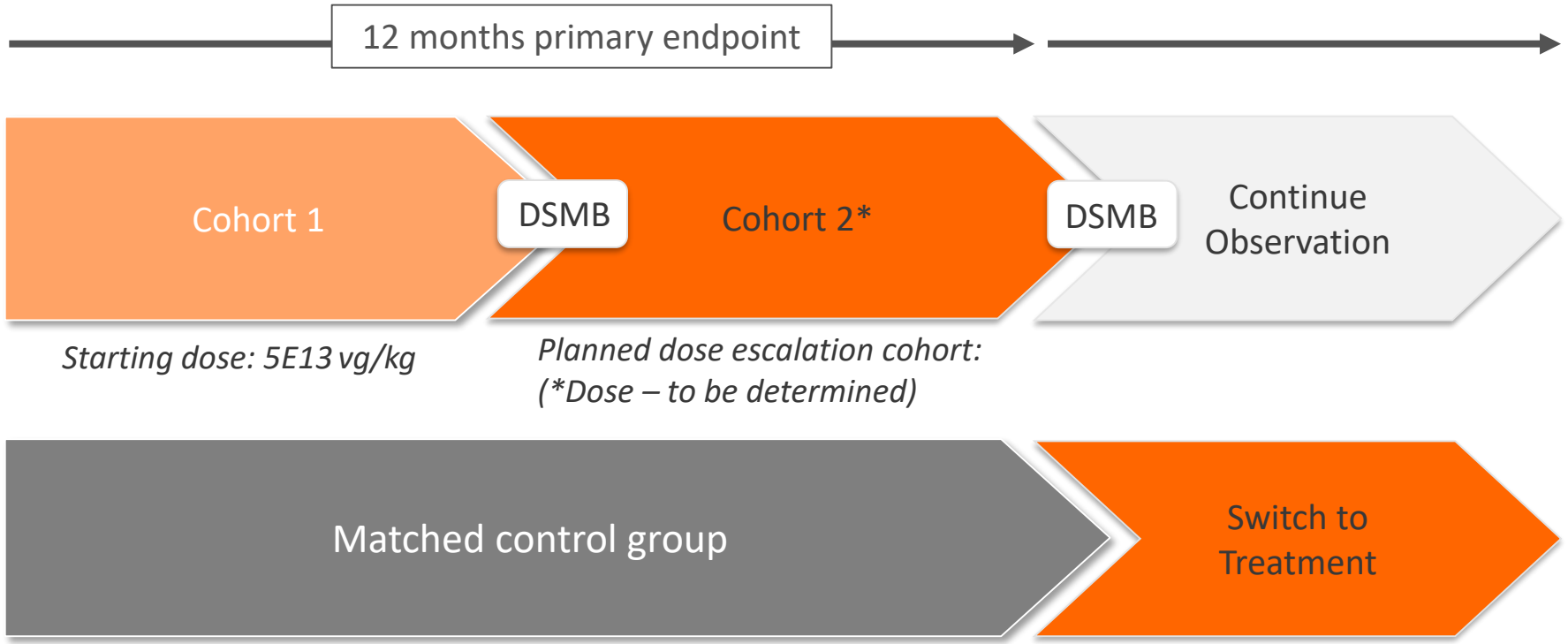
IGNITEDMD

Ambulatory children, aged 4-11 yrs

AND

Non-ambulatory adolescents, aged 12-17

(n=16 to 32)



Primary Endpoints:

- Safety & tolerability
- SGT-001 microdystrophin expression

Secondary Endpoints:

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

Visual representation only

IGNITE-DMD – Clinical Trial Update

- Dosed the first patient in February 2018
- To date, six (6) patients have been enrolled in IGNITE DMD at University of Florida
  - Three (3) to the active treatment group (5E13 vg/kg) and,
  - Three (3) to the delayed treatment control group
- Safety profile of all dosed patients remains unchanged & continue in study per protocol

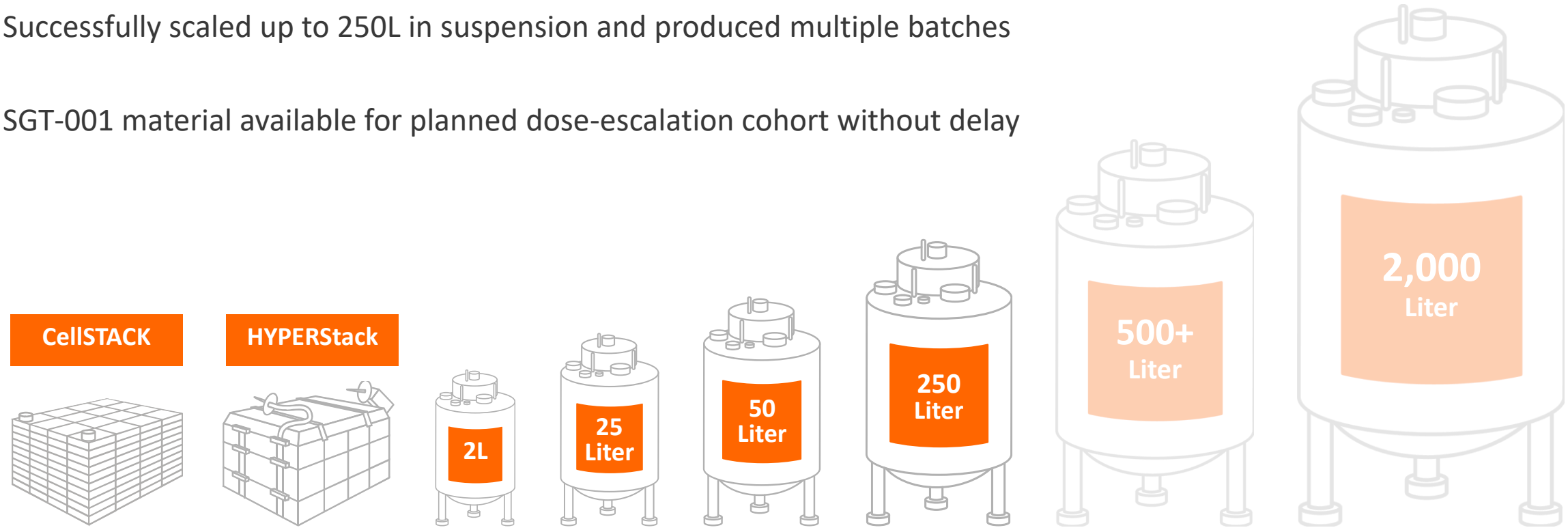
Preliminary analyses performed on the intermediate 3-month muscle biopsies in patients dosed with the starting dose of SGT-001 (5E13 vg/kg)

3-month Biopsy Data Summary

- All three biopsies showed low levels of microdystrophin protein expression via immunofluorescence
- In one patient, microdystrophin was detected via western blot (<5%) and in ~10 percent of fibers via immunofluorescence.
  - Evidence of co-localization of neuronal nitric oxide synthase (nNOS) and beta-sarcoglycan associated with microdystrophin expression

GMP Manufacturing Currently Capable of Supplying Clinical Study

- Successfully scaled up to 250L in suspension and produced multiple batches
- SGT-001 material available for planned dose-escalation cohort without delay



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

SOLID BIOSCIENCES

Thank You

