



IGNITE DMD Study of SGT-001 Microdystrophin Gene Therapy for DMD: Long-Term Outcomes and Biomarker Update

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Financial Information and 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 plans to present data from IGNITE DMD, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 or SGT-003 in patients with Duchenne, the Company’s regulatory plans, the Company’s SGT-003 program, including the Company’s expectation for filing an IND, timelines, the sufficiency of the Company’s cash, cash equivalents and available-for-sale securities to fund its operations, 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 successfully implement its headcount reduction and reduce expenses; the impact of the headcount reduction on the Company’s business; risks associated with the Company’s ability to continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; obtain and maintain the necessary approvals from investigational review boards at IGNITE DMD clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll additional patients in IGNITE DMD and on the timeline expected; the Company’s dosing strategy; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; whether the interim data referenced in this release will be predictive of the final results of the trial or will demonstrate a safe or effective treatment benefit of SGT-001 or SGT-003; whether the methodologies, assumptions and applications the Company utilizes to assess particular safety or efficacy parameters will yield meaningful statistical results; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully transition, 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 Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, SGT-003 and other product candidates, 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 press release 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.

Duchenne Muscular Dystrophy Is a Devastating Muscle-Wasting Disease



Caused by Mutations in the *DMD* Gene



1:3500-5000 Newborn Males Affected



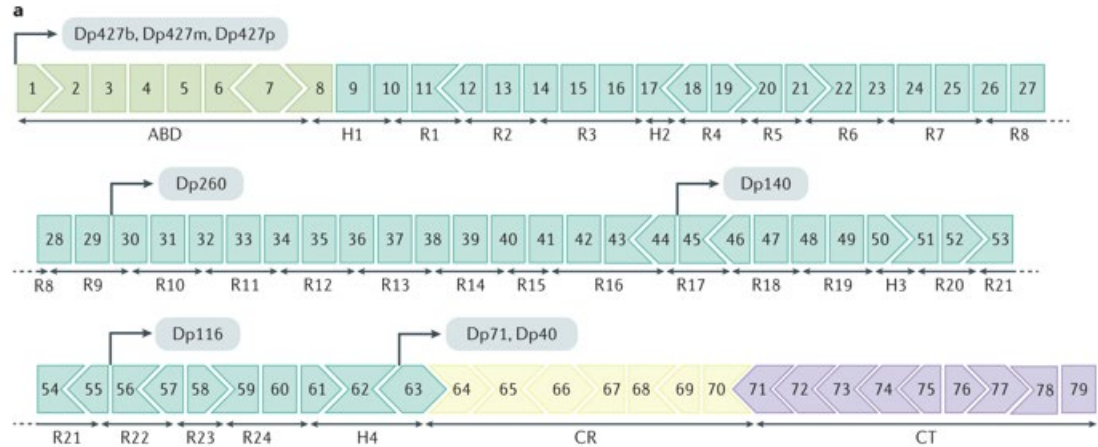
Skeletal and Cardiac Muscle Manifestations



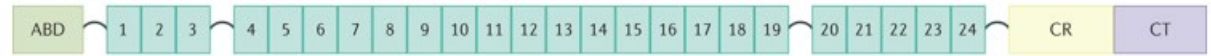
Progressive & Irreversible



No Meaningful Treatment Options



b Normal dystrophin: ABD connects to extracellular matrix with CR domain



c Duchenne muscular dystrophy: dystrophin cannot fulfil linker function because cysteine domain is lacking



d Becker muscular dystrophy: dystrophin is partially functional, crucial domains are present

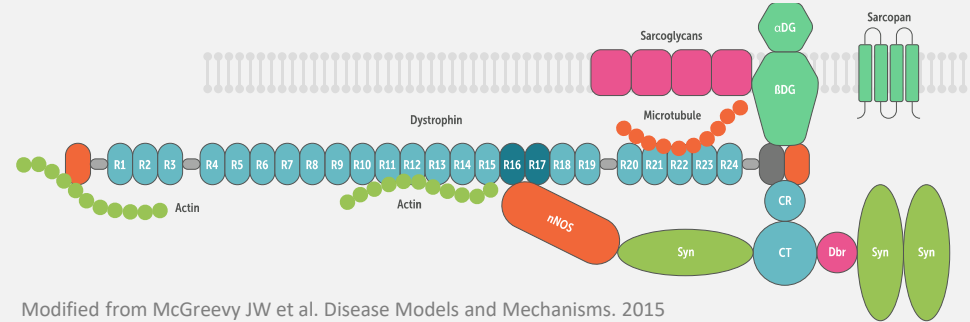


Schematic depiction of DMD gene and dystrophin protein. Duan et al. Nature Reviews Disease Primers 2021

SGT-001 Microdystrophin Gene Therapy to Replace Absent Dystrophin

Dystrophin and the Glycoprotein Complex

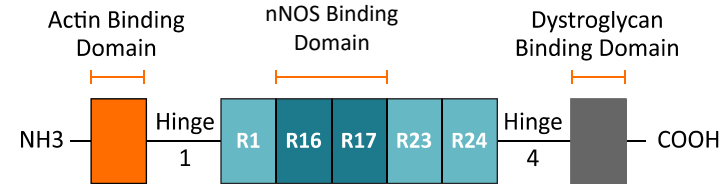
- Stabilizes the muscle membrane
- Acts as a molecular shock absorber
- Prevents muscle tissue damage and death
- Absent in Duchenne muscular dystrophy (DMD)



SGT-001: AAV9-CK8-Microdystrophin

- AAV gene transfer therapy
- Systemically delivers a unique rationally designed microdystrophin
 - Shortened form of dystrophin 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

nNOS Microdystrophin Retains key dystrophin protein functional domains



nNOS: neuronal nitric oxide synthase



**SGT-001 /
IGNITE DMD**

IGNITE DMD Study Design

Cohorts

- n=3 subjects analyzed as controls
- n=3 subjects at 5E13 vg/kg
- n=3 subjects at 2E14 vg/kg
 - 3 additional subjects dosed at 2E14 vg/kg

Key Inclusion Criteria

- Ambulatory children; mutation agnostic
- Ages 4-17 years; upper weight limits imposed throughout the study, ending with 30 kg
- Anti-AAV9 antibodies below protocol-specified thresholds
- For more information, please visit clinicaltrials.gov
NCT03368742

Primary Endpoints (Baseline to 1 Year):

- Incidence of adverse events
- Change in microdystrophin protein levels in muscle biopsies by Western blot

Select Secondary Endpoints (Baseline to 1 Year):

- 6-Minute Walk Test (6MWT)
- North Star Ambulatory Assessment (NSAA)
- Pulmonary Function Tests (PFTs)
- Quality of Life as measured by Pediatric Outcomes Data Collection Instrument (PODCI)

Enrollment in IGNITE DMD has concluded
Study participants dosed with SGT-001 will continue to be monitored for 5 years post-treatment

Overview of IGNITE DMD Safety Findings

Most Common Drug Related Clinical Adverse Reactions*

Nausea	(9/9)
Vomiting	(8/9)
Fever	(7/9)

- The most common drug related laboratory abnormalities were thrombocytopenia/decreased platelets, anemia, proteinuria, and increases in fibrin, D dimer, soluble C5b9 and LDH**
- Activation of the terminal pathway (sC5b9) of the classical complement system occurred in all subjects resulting in 3 serious adverse events (SAEs): Systemic Inflammatory Response Syndrome (2); thrombocytopenia (1)
- Two other SAEs: immune hepatitis 4 weeks post dosing which resolved rapidly after a transient increase of corticosteroids (1); Giardiasis, determined to be unrelated to SGT-001 (1)
- All SAEs are resolved

All patients continue to be monitored with follow-up periods ranging from >6 months to approximately 4 years post-dosing. No treatment-associated AEs have occurred in any subject after 90 days post-infusion

*Less common adverse reactions include cytokine release syndrome, generalized edema, acute kidney injury and thrombotic microangiopathy

**Less common laboratory abnormalities include increased CPK, decreased complement, increased liver enzymes, increased troponin, decreased hemoglobin, increased haptoglobin urinary casts and leukocytosis

Durable Microdystrophin Expression and Protein Function in Biopsies Collected at Timepoints Ranging from 12-24 Months Post-Dosing

Range of 3-Month Microdystrophin Expression Patients 4-9		
	% Positive Fibers (Immunofluorescence)	% of Normal Dystrophin (Western Blot)
Pt 4-9	1% to 70%	BLQ to 17.5%

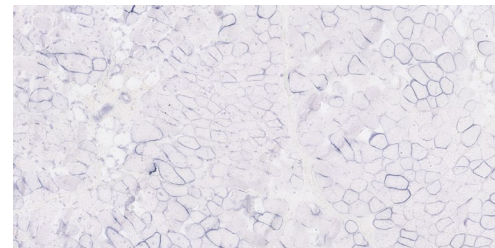
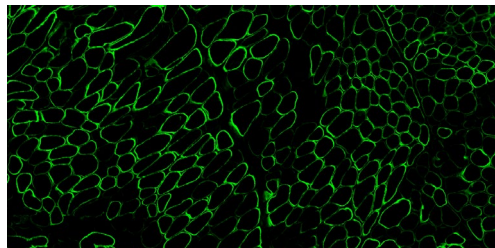
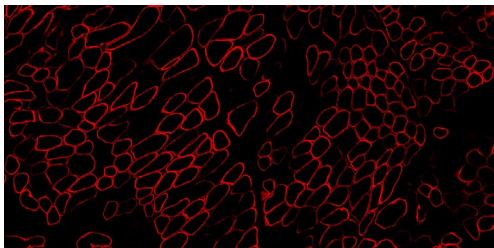
Long-Term (12-24 Months) Microdystrophin Expression Patients 4-6		
	% Positive Fibers (Immunofluorescence)	% of Normal Dystrophin (Western Blot)
12m (n=1)	50-60%	20.3%
18m (n=1)	85%	69.8%
24m (n=1)	10-30%	BLQ

Microdystrophin

β -Sarcoglycan

nNOS Activity

Pt 5
(18 months)



Limited Dystrophic Pathology Progression Over 12-24 Months

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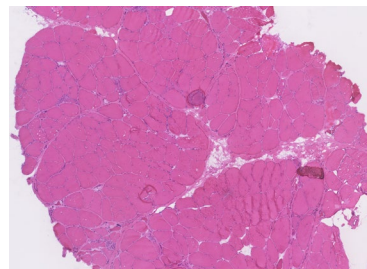
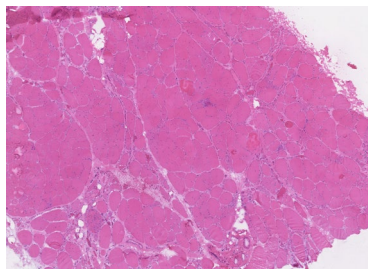
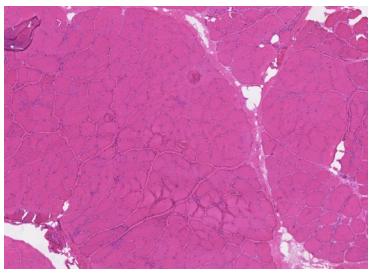
Baseline

3 Months

Last Timepoint

Pt 4

(Age at Dosing: 10.7 yrs)

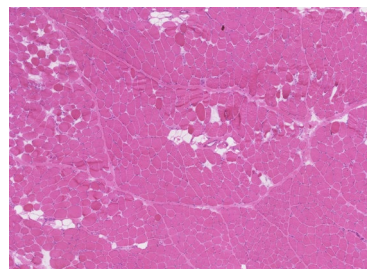
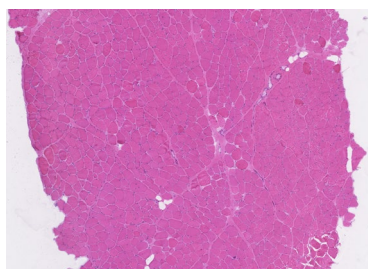
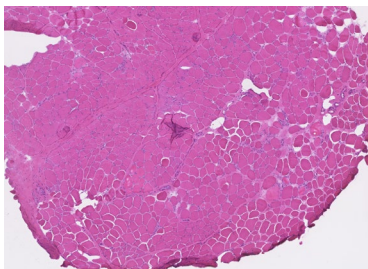


24-Month Biopsy
(Age at Biopsy: 12.7 yrs)

Very mild active dystrophic pathology

Pt 5

(Age at Dosing: 6.9 yrs)

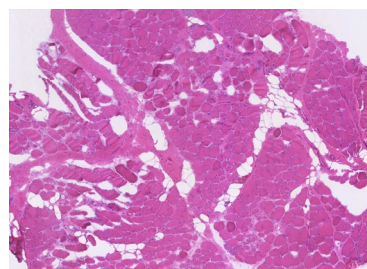
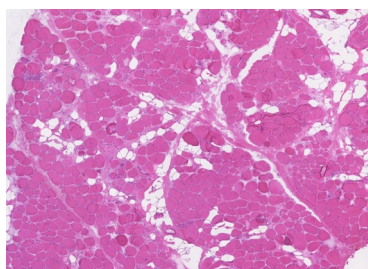
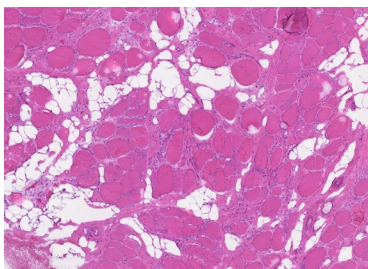


18-Month Biopsy
(Age at Biopsy: 8.3 yrs)

No active dystrophic pathology

Pt 6

(Age at Dosing: 7.7 yrs)

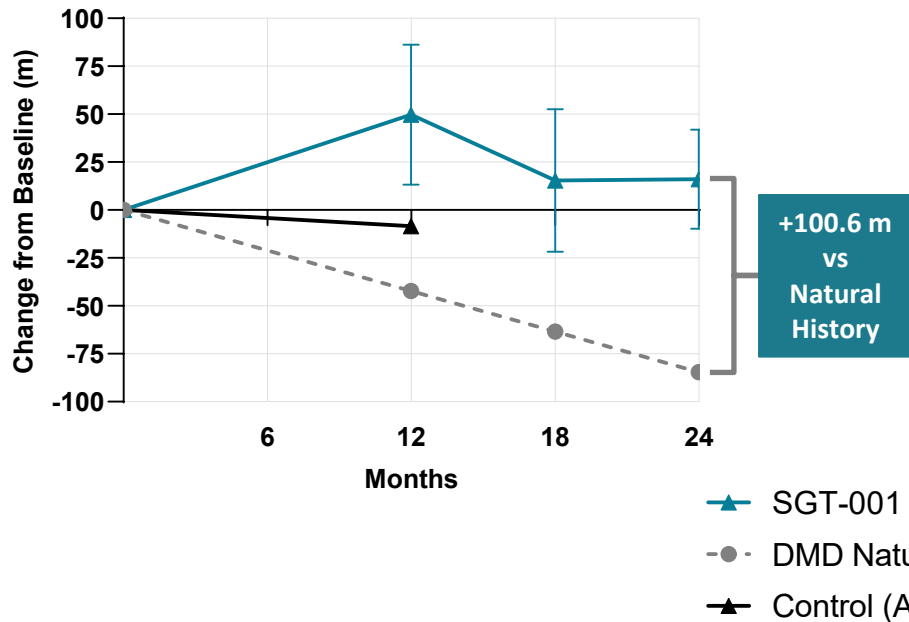


12-Month Biopsy
(Age at Biopsy: 8.7 yrs)

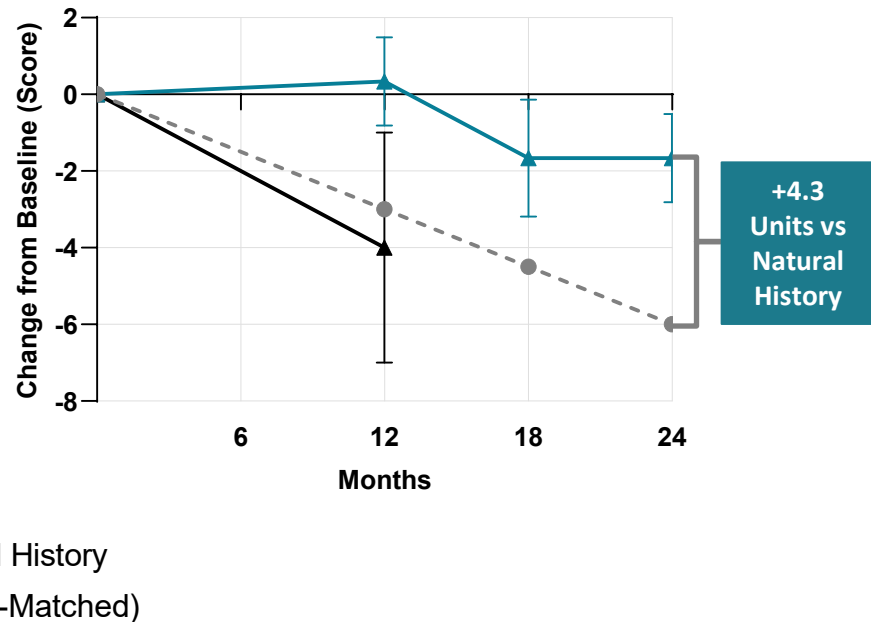
Very mild active dystrophic pathology

SGT-001 Treated Patients 4-6 Continue to Show Consistent, Stable Motor Function by 6MWT and NSAA at 2 Years Post-Dosing Compared to Natural History

6 Minute Walk Test (6MWT)



North Star Ambulatory Assessment (NSAA)

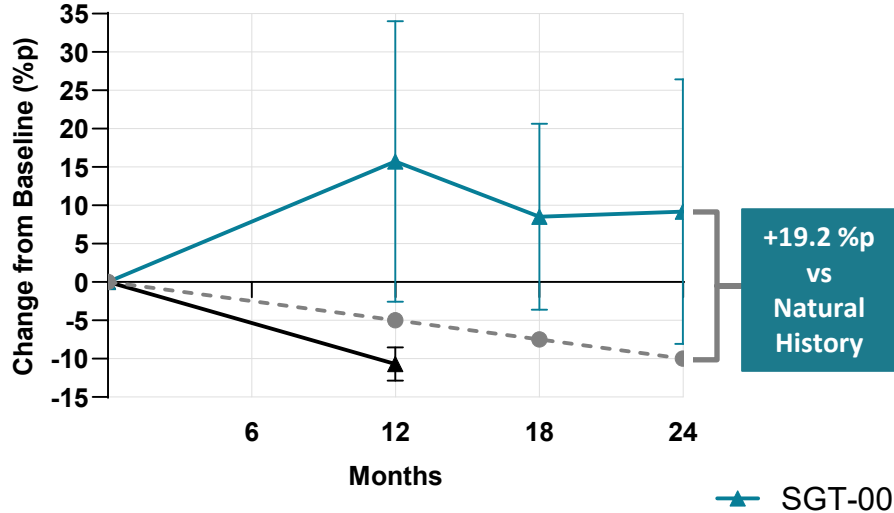


-84.6 m expected decline in 24 months after age 7 (Mercuri et al 2016)

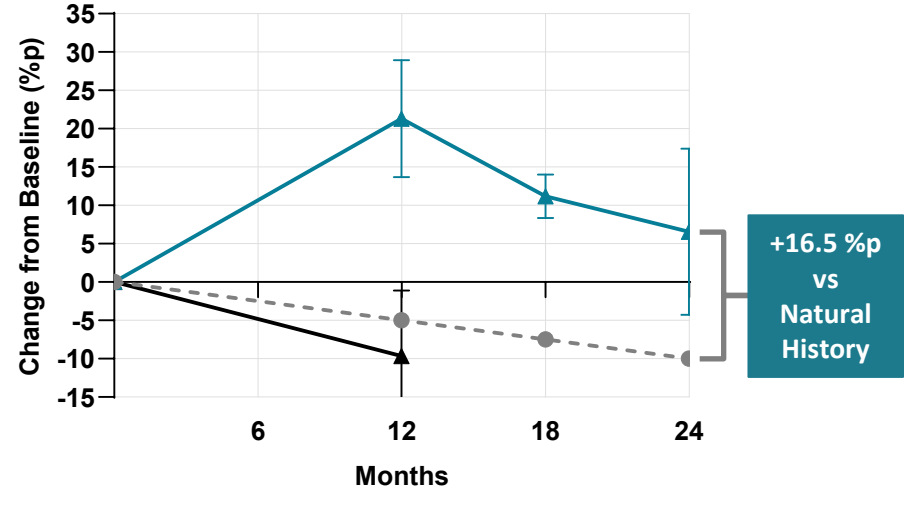
-6.0 unit expected decline in 24 months after age 6.3 (Muntoni et al 2019)

Pulmonary Function Tests Show Durable Improvements in SGT-001 Treated Patients 4-6 across 2 Years after Dosing

Forced Vital Capacity % Predicted (FVC %p)



Peak Expiratory Flow % Predicted (PEF %p)

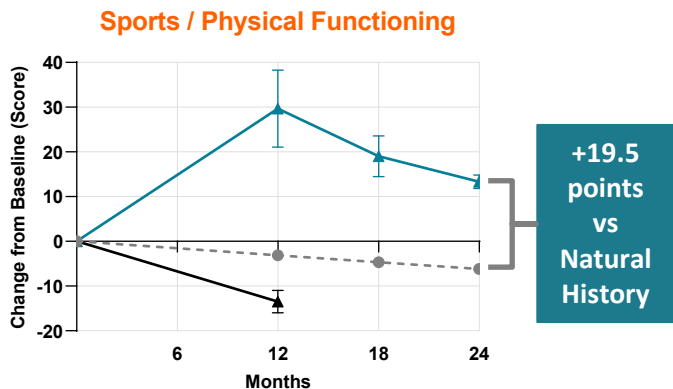
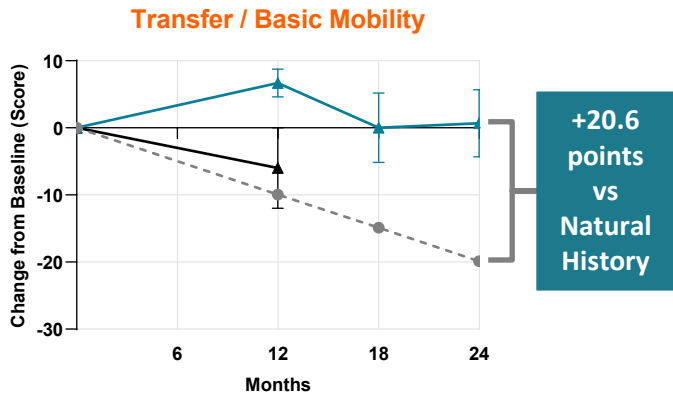
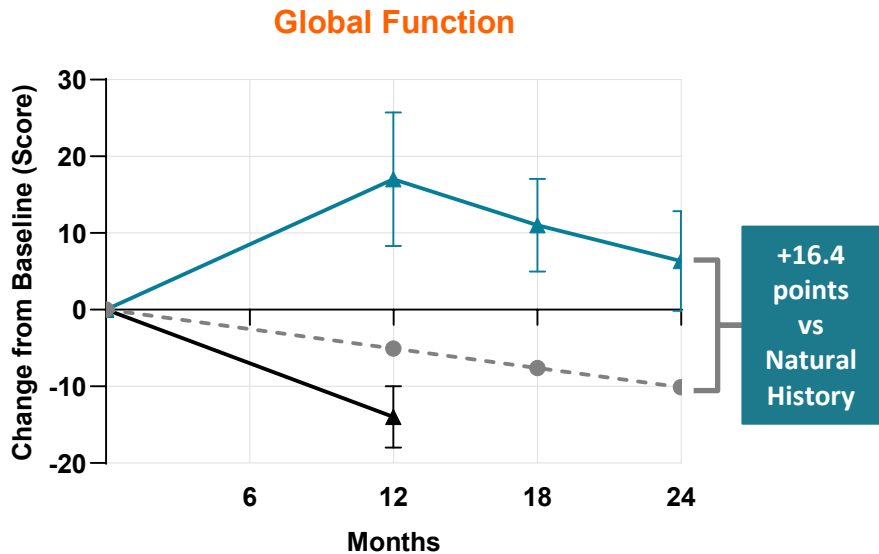


- ▲ SGT-001
- DMD Natural History
- ▲ Control (Age-Matched)

-10.0 %p expected decline in 24 months after age 6 (Mayer et al 2015)

-10.0 %p expected decline in 24 months after age 6 (Mayer et al 2015)

SGT-001 Treated Patients 4-6 Report Stability or Improvements in Key Functional Domains of the PODCI after 2 Years



Expected declines in 24 months (Henricson et al 2013)

- Global: -10.1 points
- Transfer / Basic Mobility: -19.9 points
- Sports / Physical Functioning: -6.2 points

- ◆ SGT-001
- DMD Natural History
- ▲ Control (Age-Matched)

Key Takeaways From Interim Analysis of IGNITE DMD



Sustained motor function

- ✓ Stable 6 Minute Walk Test (6MWT) distances and North Star Ambulatory Assessment (NSAA) scores compared to natural history



Improved pulmonary function

- ✓ Improvements in Forced Vital Capacity (FVC %p) and Peak Expiratory Flow (PEF %p) compared to baseline and natural history



Continued meaningful improvements in patient reported outcomes

- ✓ Stable or improved scores across functional domains of the PODCI compared to baseline and natural history



All patients dosed with SGT-001 in the high dose cohort have demonstrated microdystrophin expression and localization

- ✓ 90-day biopsy data from Patients 7-9 within the range of expression for Patients 4-6
- ✓ Long-term biopsy data from Patients 4-6 demonstrate durable microdystrophin expression at 12-24 months post-dosing

SGT-001 treated patients show consistent, durable improvements in function across assessments 2 years after dosing compared to expected natural history declines

SGT-003 AND NOVEL CAPSIDS

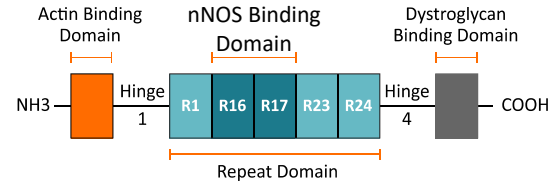


SGT-003 is Rapidly Advancing to the Clinic Using a Next Generation Muscle-Tropic Capsid



Novel Capsid

- Rational design of next-generation novel capsid
- Demonstrated differentiated muscle tropism with improved efficacy per dose compared to AAV9



nNOS Microdystrophin

- Continue to deliver best-in-class, optimized microdystrophin construct with nNOS domain
- Skeletal and cardiac muscle expression driven by the muscle-specific CK8 promoter

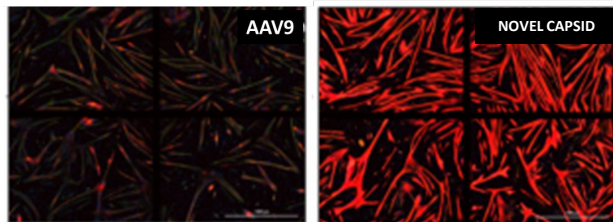
Data from the IGNITE DMD clinical study and preclinical data on novel capsid support continued SGT-003 development

Compelling Data Resulted in Advancement of SGT-003 Program

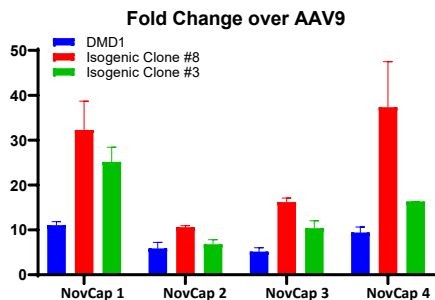
Capsid library development *in vitro* screening and *in vivo* testing

Cell Based *In Vitro* Assays WT Mouse and Human Dystrophic Cells

C2C12 Microdystrophin Protein Expression

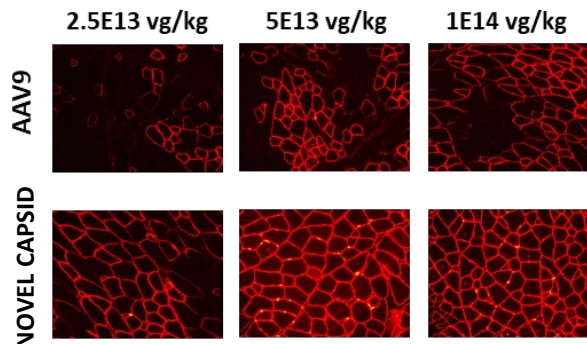


Human DMD Cell Microdystrophin Expression

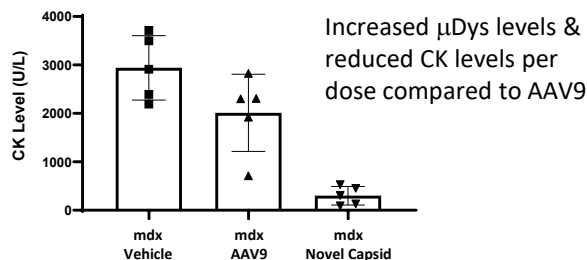


In Vivo Disease Model (mdx) Testing

IF Imaging of mdx Quads



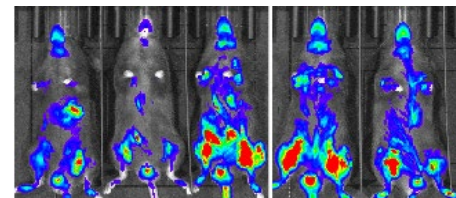
Serum CK Day 29 Post-Treatment



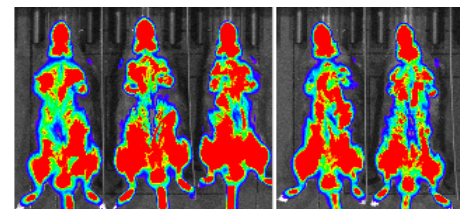
Interim data

In Vivo WT Mouse Reporter

AAV9 3E14 vg/kg

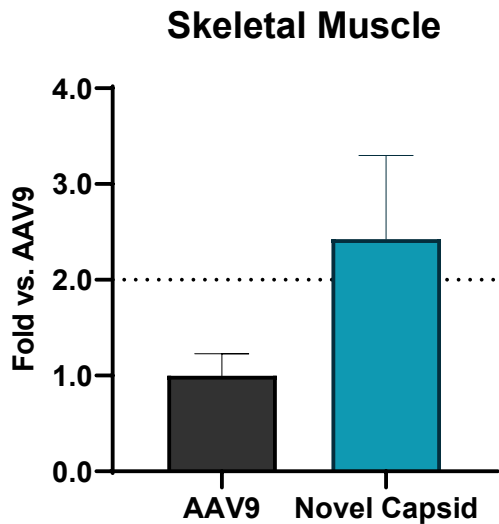


Novel Capsid 3E14 vg/kg

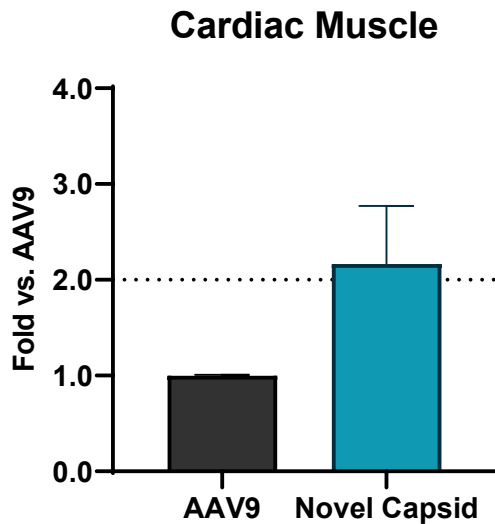


Advanced to Program status: SGT-003

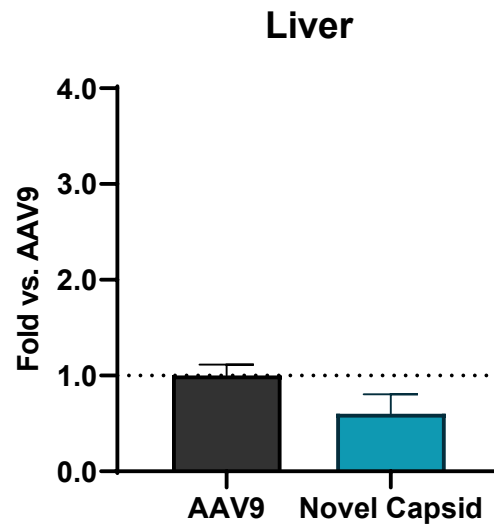
NHPs Administered Novel Capsid Show Increased Muscle Biodistribution and Decreased Biodistribution to Liver Relative to AAV9



>2X vs AAV9



>2X vs AAV9

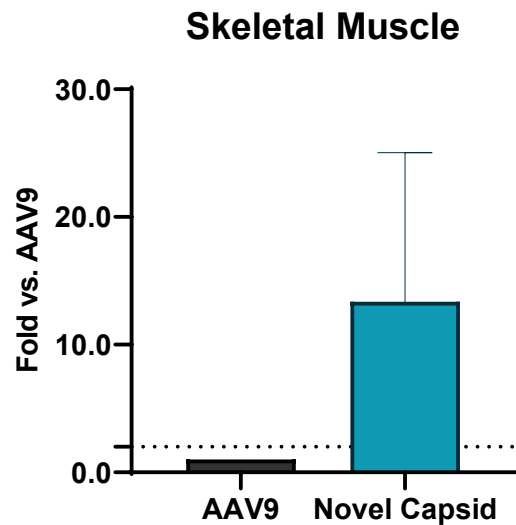


>0.5X vs AAV9

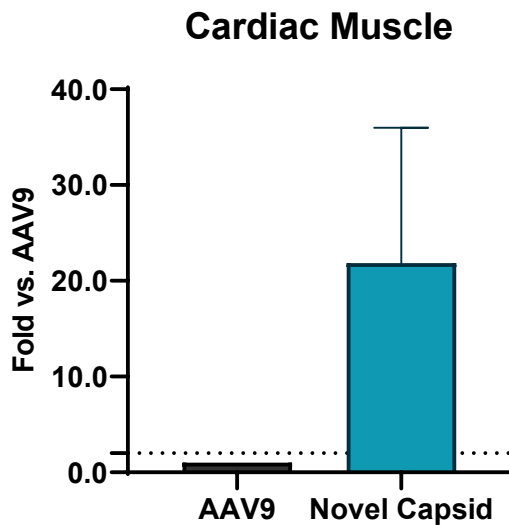
* Average fold differences calculated from four skeletal muscle tissues, three cardiac muscles and a liver sample

Luciferase Expression Data Support Relative Differences Observed in Biodistribution between Novel Capsid and AAV9

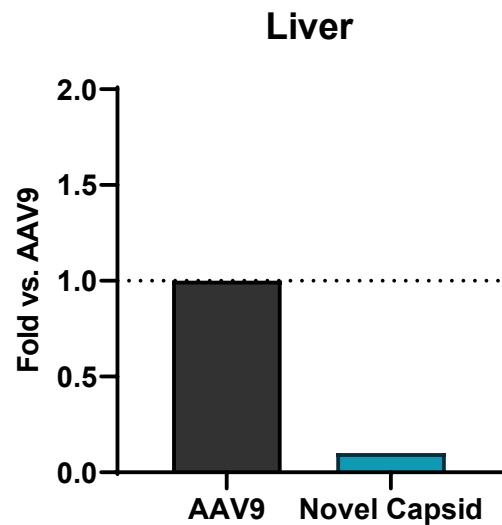
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>10X vs AAV9



>20X vs AAV9



<0.5X vs AAV9

* Average fold differences calculated from five skeletal muscle tissues, three cardiac muscles and a liver sample

Solid's Path Forward for SGT-001 and SGT-003

Development Activities

SGT-001

- Enrollment in IGNITE DMD has concluded
- Activities transitioning to FDA engagement
- Dosed patients to be monitored for 5 years post-treatment and have functional data shared
- Solid to refine clinical development strategy with future patients being dosed with SGT-001 produced via transient transfection

SGT-003

- Advancing rapidly to bring a next-generation AAV microdystrophin gene transfer therapy to the clinic next year
- Translatability activities across species continue
- Evaluation of exploratory studies and other research activities are ongoing that could be applicable to other muscle-related disorders

Natural History study – add to clinical understanding of Duchenne

Exploring alternative immunosuppression strategies for future clinical studies



Thank You

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