IGNITE DMD Phase I/II Study of SGT-001 Microdystrophin Gene Therapy for DMD: 2-Year Outcomes Update

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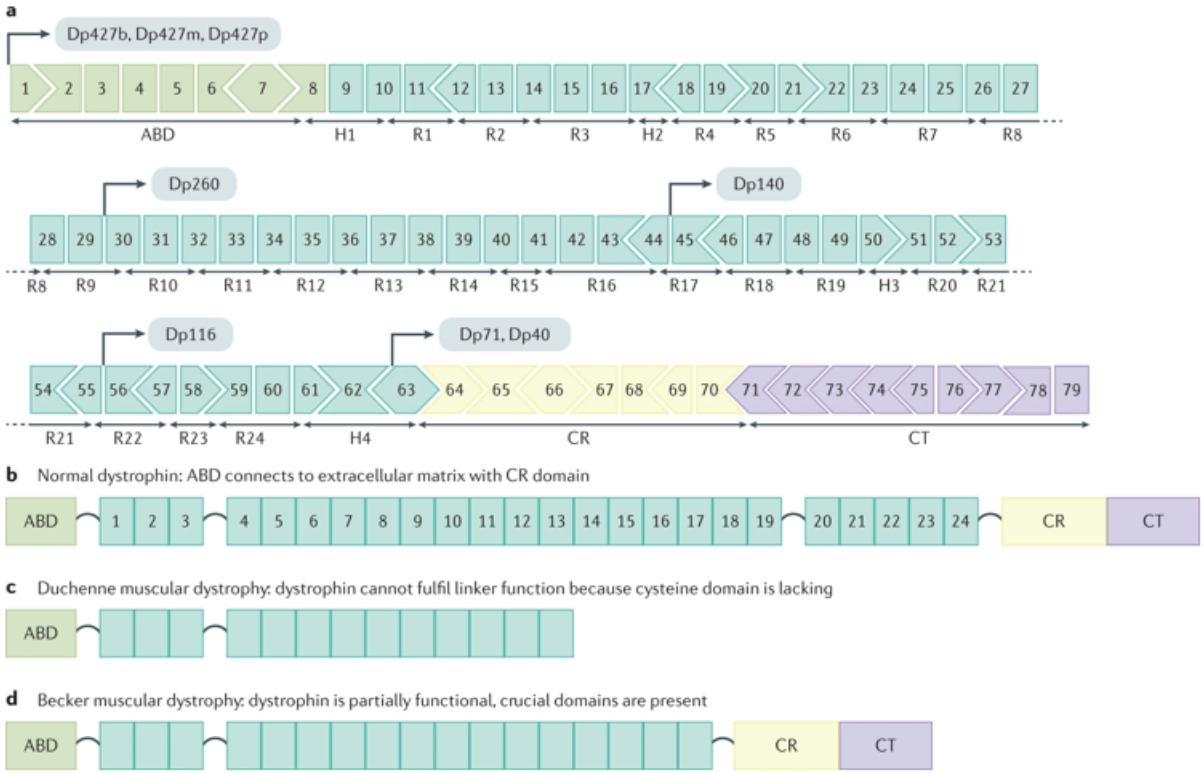
MDA Clinical and Scientific Conference 2022

Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the ability of the Company to continue dosing patients in the IGNITE DMD trial, 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 in patients with DMD, 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 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 predicative of the final results of the trial or will demonstrate a safe or effective treatment benefit of SGT-001; 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 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
- 100% FATAL — Progressive & Irreversible
- No Meaningful Treatment Options



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

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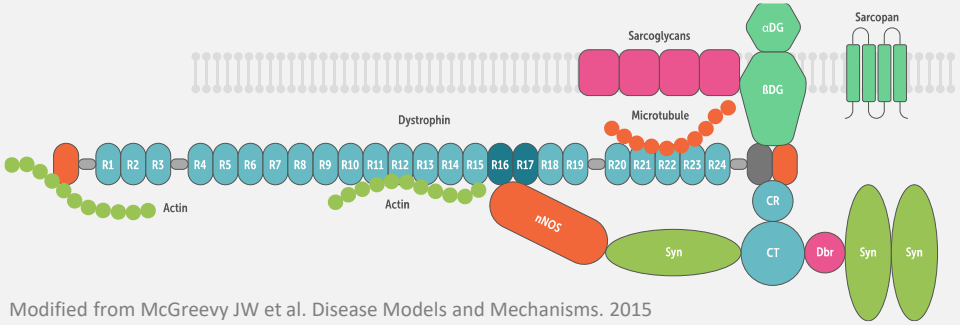
SGT-001 Microdystrophin Gene Therapy to Replace Absent Dystrophin

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)

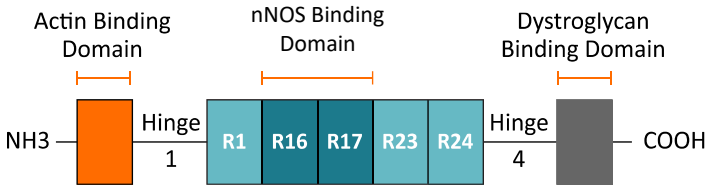


Modified from McGreevy JW et al. Disease Models and Mechanisms. 2015

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

SGT-001 Microdystrophin
Retains key dystrophin protein functional domains



nNOS: neuronal nitric oxide synthase

IGNITE DMD Study Design: *Two Dose Levels Initially Assessed; 2E14 vg/kg Selected*

Interim Analysis of Subjects in IGNITE DMD

- 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

Inclusion Criteria

- Ambulatory children; mutation agnostic
- Ages 4-17 years; upper weight limit of 18 kg for next two patients dosed; up to 30 kg (~66 lbs) for remainder of the clinical trial
- Primary focus on children with the potential to include adolescent patient population in the future
- 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):

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

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 4 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

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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
(Updated 14 Mar 2022)

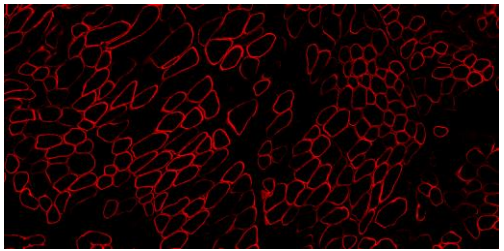
	% Positive Fibers (Immunofluorescence)	% of Normal Dystrophin (Western Blot)
Pt 4-9	1% to 70%	BLQ to 17.5%
Pt 7-9	1% to 50%	BLQ to 6.8%

Long-Term (12-24 Months) Microdystrophin Expression
Patients 4-6

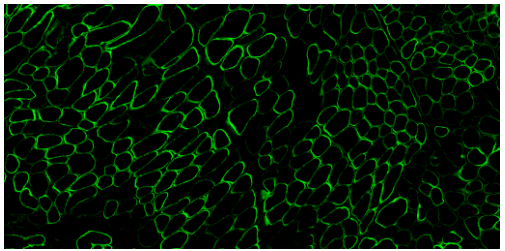
	% Positive Fibers (Immunofluorescence)	% of Normal Dystrophin (Western Blot)
12m (n=1)	50-60%	20.3%
18m (n=1)	85%	69.8%
24m (n=1)	10-30%	BLQ

Pt 5
(18 months)

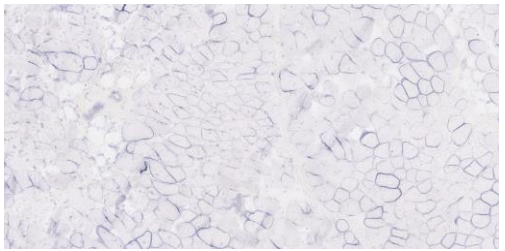
Microdystrophin



β-Sarcoglycan

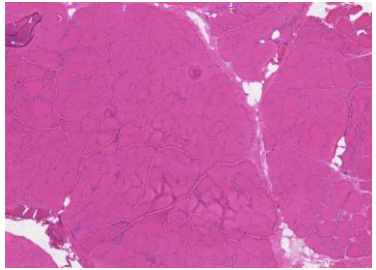
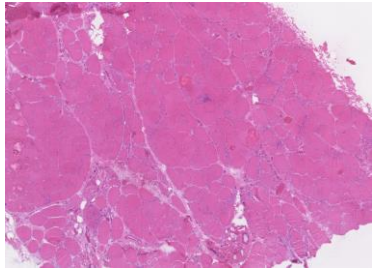
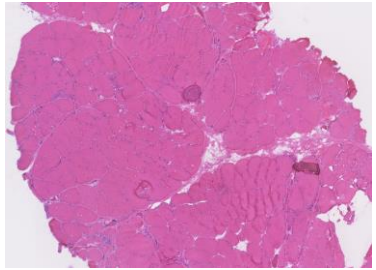
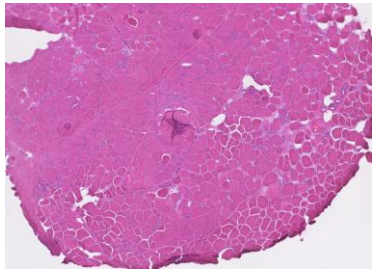
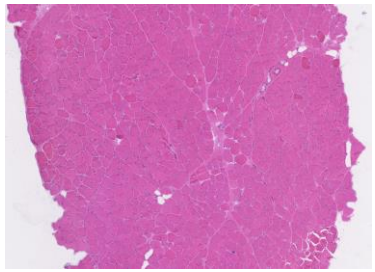
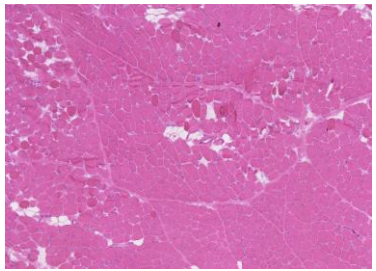
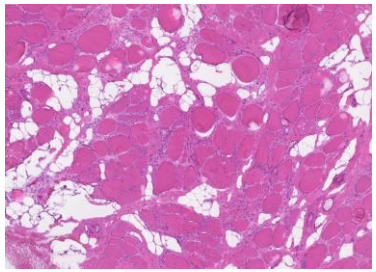
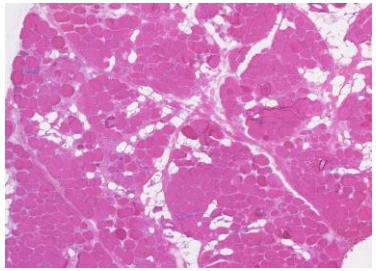
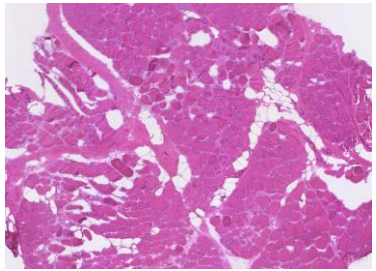


nNOS Activity



BLQ: Below the 5% limit of quantification

Limited Dystrophic Pathology Progression Over 12-24 Months

	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*

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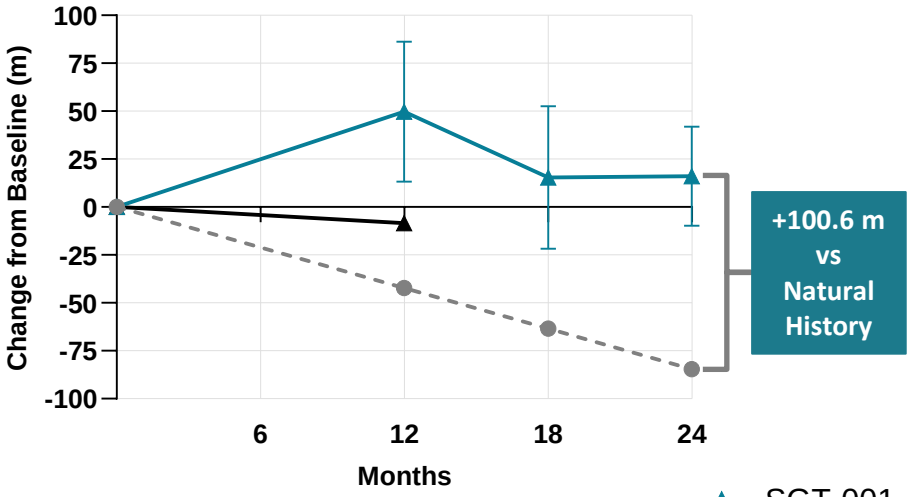
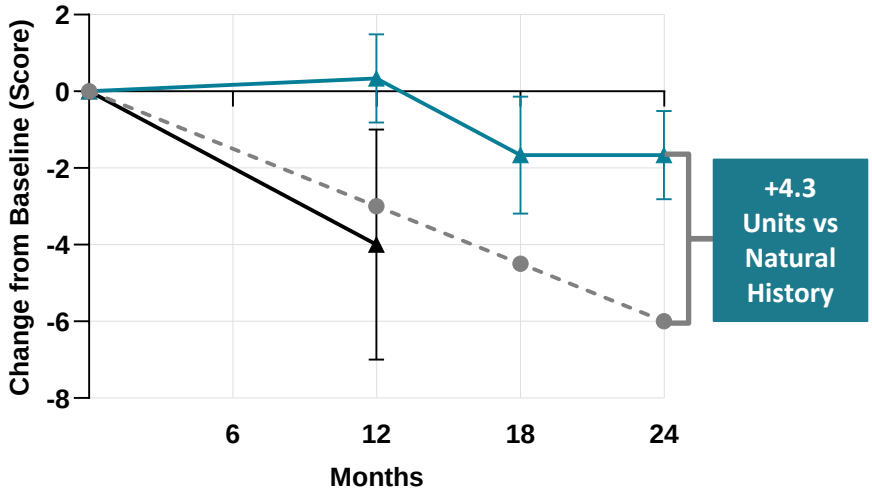
SGT-001 Treated Patients 4-6 Continue to Show Consistent, Stable Motor Function by 6MWT and NSAA at 2 Years Post-Dosing Compared to Natural History

6 Minute Walk Test (6MWT)



North Star Ambulatory Assessment (NSAA)



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

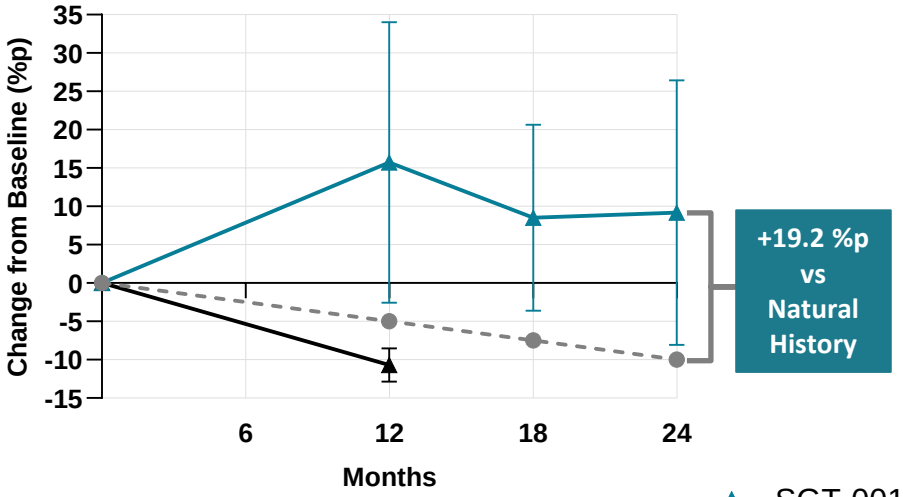
-84.6 m expected decline in 24 months after age 7 (Mercuri et al 2016)

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

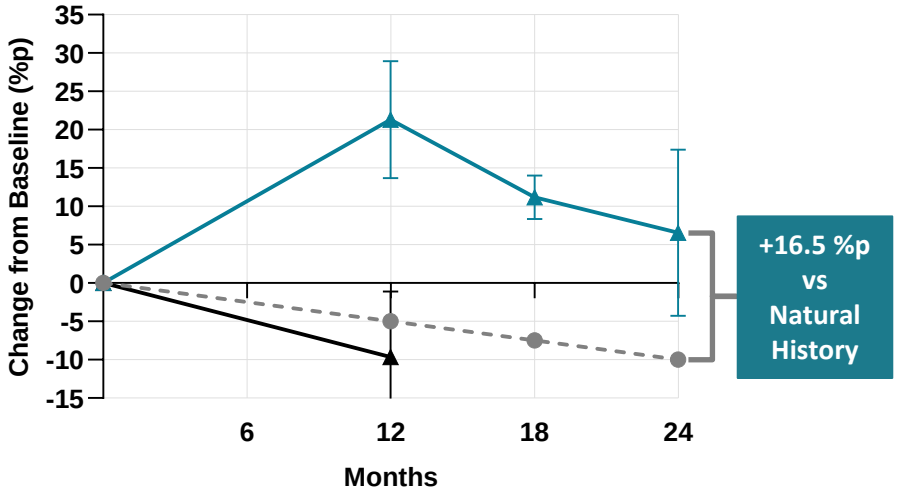
Interim data
Mean calculation based on IGNITE DMD Patients 4-6

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)

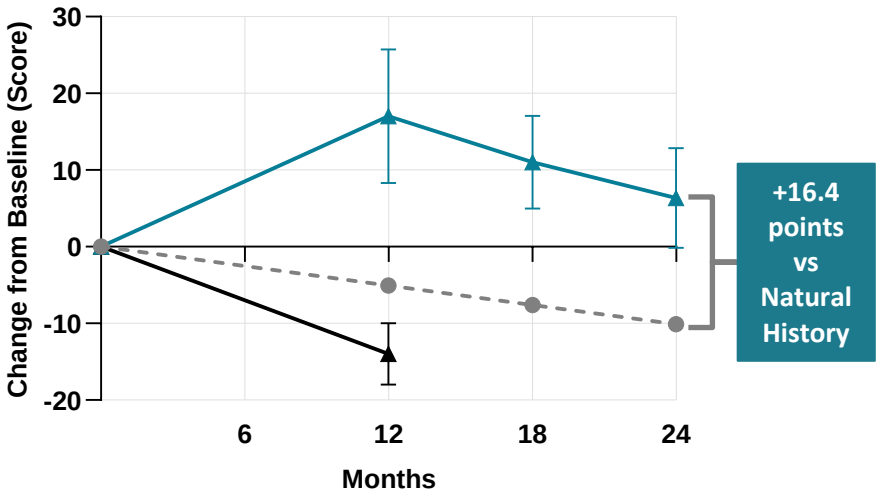
-10.0 %p expected decline in 24 months after age 6 (Mayer et al 2015)

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

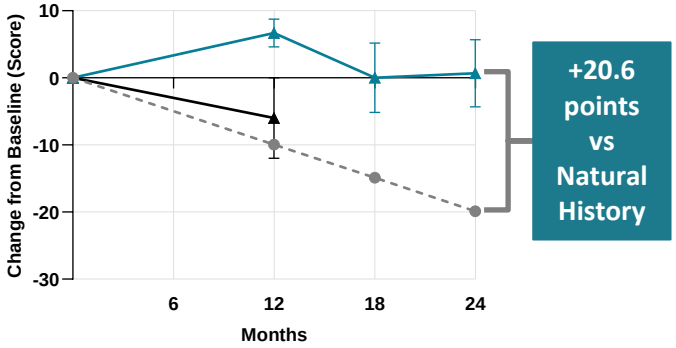
Interim data
Mean calculation based on IGNITE DMD Patients 4-6

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

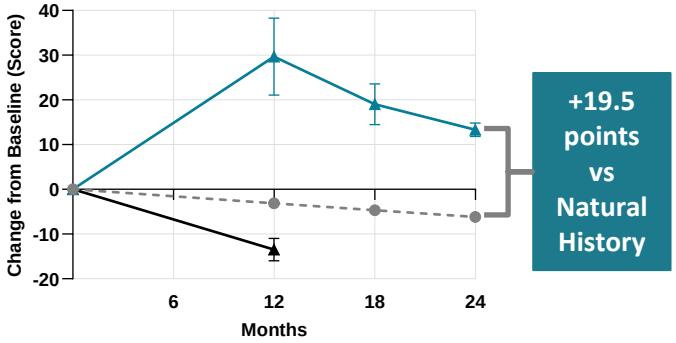
Global Function



Transfer / Basic Mobility



Sports / Physical Functioning



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)

Interim data
Mean calculation based on IGNITE DMD Patients 4-6

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

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