SGT-001 Interim Clinical Results From IGNITE-DMD

Ilan Ganot, Co-founder, President, and CEO

March 15, 2021

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, ability of the Company to continue dosing patients in the IGNITE DMD trial, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 in patients with Duchenne, the Company’s expectations for reporting future data from the IGNITE DMD trial, the Company’s regulatory plans and timelines 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 clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll patients in IGNITE DMD; 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 presented 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 we utilize 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 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.

Introductions

Ilan Ganot

Co-Founder, President and Chief Executive Officer

Joel Schneider, PhD

Chief Operating Officer

Cathryn Clary, MD, MBA

Acting Chief Medical Officer

Carl Morris, PhD

Chief Scientific Officer

Barry Byrne, MD,PhD

Assoc. Chair of Pediatrics and Director of the Powell Gene Therapy Center at the University of Florida; Principal Investigator of IGNITE DMD

Key Accomplishments in 2020 Set the Stage for 2021 and Beyond

Enrollment in IGNITE-DMD resumed

Ultragenyx partnership formed; additional program added to Solid pipeline

Investment in SGT-001 manufacturing and ongoing operations

2021 Priorities and Anticipated Milestones

Resume dosing patients in IGNITE DMD
(Q1 2021)

Present 12-month safety & efficacy for patients 1-6
(Q1 2021)

Present 90-day biopsy data for additional patients dosed in IGNITE DMD
(2H 2021)

Advance towards commercial readiness

Prepare for registration study

Further pipeline expansion

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IGNITE DMD Design & Clinical Outcomes

Cathryn Clary, MD, MBA, Acting Chief Medical Officer

IGNITE-DMD Interim Results

Study Overview

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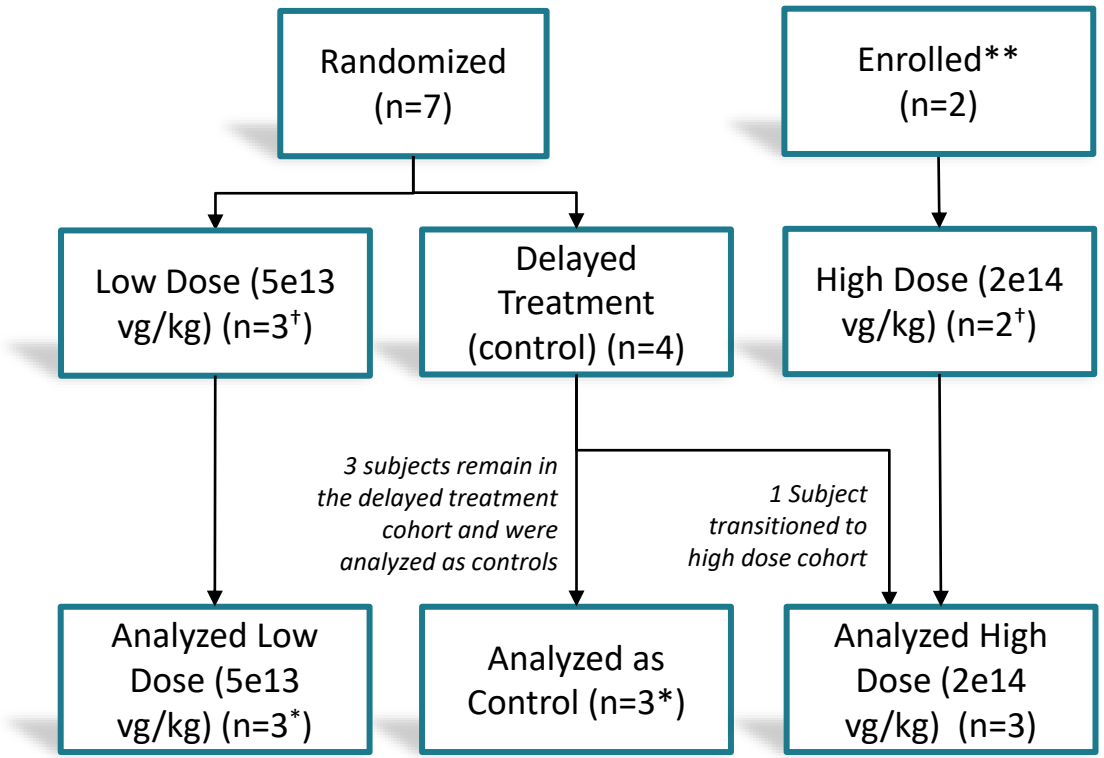
IGNITE-DMD Study Design:

Two Dose Levels Initially Assessed; 2e14 vg/kg Selected

Interim Analysis of Subject in IGNITE DMD*



* Does not include Patient 7.

**After initial randomization into either low dose or delayed treatment, the dose was escalated to 2e14 vg/kg based on biopsy results from 5e13 vg / kg cohort per protocol

† includes one non-ambulatory subject, each

Population

Males aged 4 to 17 with documented dystrophin gene mutation predictive of DMD*

4 years to 11 years (inclusive):

- Walk & climb stairs with/without aid of railing (Vignos scale score ≤ 3)
- Rise from the floor from supine (Gowers) time ≤ 7 seconds

12 years to 17 years (inclusive):

- Unable to walk 10 meters without assistance
- Score ≤ 4 on Brooke scale for upper extremity

A stable dose of corticosteroids of ≥0.5 mg/kg/day of oral prednisone or equivalent for ≥ 12 wks prior to dosing. Expected to remain constant throughout the study on a per kg basis, except for protocol-specified dose changes

Primary Endpoints (Baseline to One Year):

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

Select Secondary Endpoints (Baseline to One Year):

- North Star Ambulatory Assessment (NSAA) total score
- Six Minute Walk Distance
- Pulmonary Function Tests
- Quality of Life as measured by Pediatric Outcomes Clinical Instrument and Modus Outcomes

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Demographic and Baseline Characteristics of Subjects Analyzed in Interim Analysis

	Control Cohort			Low Dose Cohort 5e13 vg/kg			High Dose (2E14) Cohort 2e14 vg/kg		
	CT 1	**CT 2**	**CT 3**	**PT1**	**PT2**	**PT3**	**PT4**	**PT5**	**PT6***
Age at baseline	15.3	9.5	6.2	14.4	5.2	6.9	10.7	6.8	7.7
Age at 1 year	16.4	10.6	7.3	15.4	6.2	7.9	11.7	7.9	8.8
Age at last visit assessed	16.4	10.6	7.3	16.4	7.3	9.0	12.2	7.9	8.8

*Entered the study in the delayed treatment cohort, subsequently treated with High Dose

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Summary of Interim Efficacy Results of IGNITE-DMD

Absolute Change From Baseline to One Year

		CK (U/L)	NSAA	6MWT (m)	FVC%	PODCI – Sports	PODCI – Global
Control	CT 1 *15.3 yrs*	+2,831	n/a	n/a	-9.6%	-16	-18
	CT 2 *9.5 yrs*	-3,428	-1	-8	-7.6%	-11	-10
	CT 3 *6.2 yrs*	-3,810	-7	-9	-15.0%	n/a	n/a
5E13 vg/kg	Pt 1 *14.4 yrs*	-1,507	n/a	n/a	+8.9%	-6	+18
	PT 2 *5.2 yrs*	+14,300	-3	+12.0	+5.3%	+5	+6
	PT 3 *6.9 yrs*	+13,846	+5	+62.0	-2.4%	+21	+9
2E14 vg/kg	PT 4 *10.7 yrs*	-8,455	+1	+12.0	+3.1%	+22	+13
	PT 5 *6.8 yrs*	-8,381	-1	+85.0	+36.7%	+28	+11
	PT 6 *7.7 yrs*	-5,305	+1	+52.0	+10.2%	+39	+27

IGNITE-DMD Results

Safety, Biomarkers & Clinical Outcomes

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Endpoints Evaluated in the Interim Analysis of IGNITE-DMD

SAFETY

- Most common adverse reactions
- Most common laboratory abnormalities

FUNCTIONAL ASSESSMENTS

- North Star Ambulatory Assessment
- 6-Minute Walk Test
- Pulmonary Function Tests

BIOMARKERS

- Microdystrophin biodistribution, and localization by IF
- Microdystrophin protein expression by WB
- Creatine kinase levels

PATIENT REPORTED OUTCOMES

- Pediatric Outcomes Data Collection Instrument
- Modus Outcomes

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SAFETY

No New Drug-related Safety Findings Have Been Identified 17 to 37 Months Post Infusion. All Previously Reported SAEs Have Fully Resolved

Most Common Drug Related Clinical Adverse Reactions* (*previously reported*)	
Nausea	(6/6)
Fever	(4/6)
Vomiting	(5/6)

- The most common drug related laboratory abnormalities** were thrombocytopenia, increased fibrin D-dimer, increased soluble C5b9, increased LDH, proteinuria
- Activation of the terminal pathway (sC5b9) of the classical complement system occurred in all subjects resulting in two serious adverse events (SAEs)
- Two other SAEs included an episode of immune hepatitis 4 weeks post dosing which resolved rapidly after a transient increase of corticosteroids and one unrelated to SGT-001. All SAEs are resolved.

*Less common adverse reactions (33% incidence) include Cytokine Release Syndrome, Generalized edema, Hepatoxicity, Systemic inflammatory response, Thrombotic microangiopathy

**Less common laboratory abnormalities include: “Increased CPK, Decreased complement (50%),” “Increased LFTs, increased troponin (33%),” “Decreased HgB, increased haptoglobin urinary casts, leukocytosis, CRP elevated (17%)”

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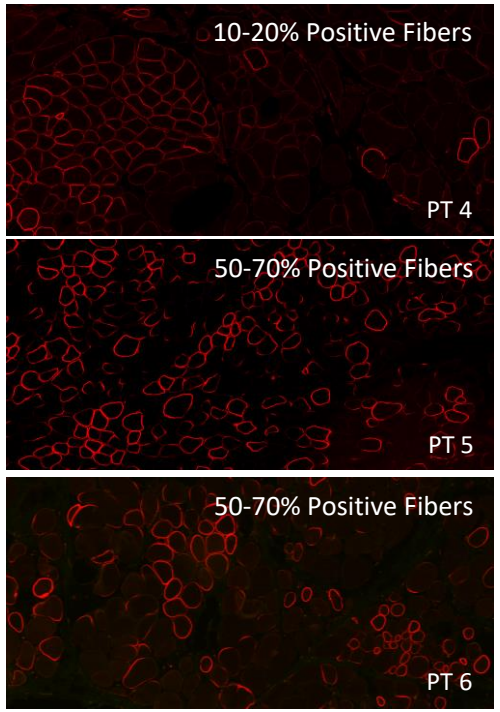
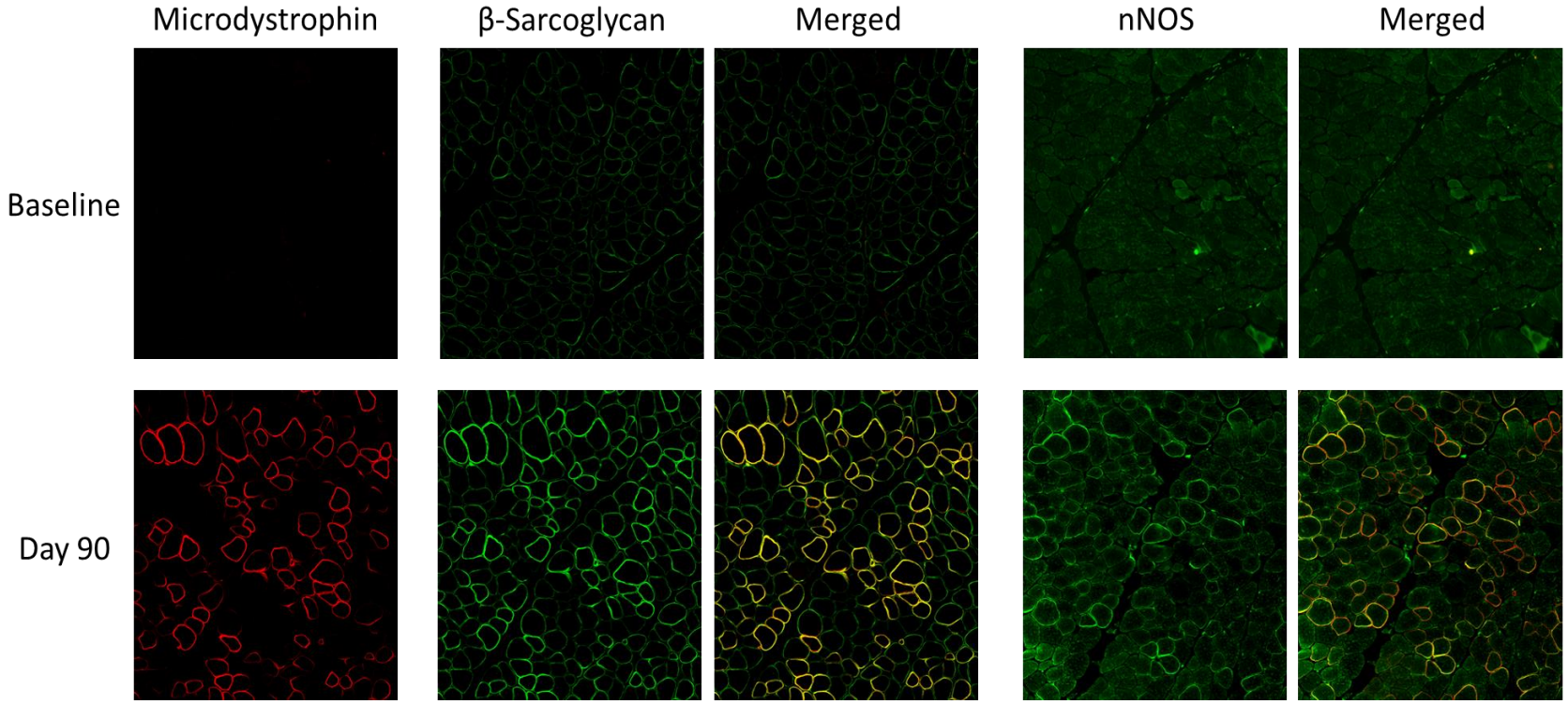
Widespread Distribution Of Microdystrophin-Positive Muscle Fibers With Co-localization Of nNOS And β-sarcoglycan In The Muscles Of Treated Subjects

Distribution of Microdystrophin in All 3 High Dose Subjects at Day 90*



Representative Histology (Patient 5) Illustrating Co-Localization of Microdystrophin, β-Sarcoglycan, and nNOS at the Muscle Membrane*



Average Microdystrophin protein level in subjects in the high dose cohort via WB at Day 90: ~10% of normal dystrophin (from ~5% to 17.5%)

*Data presented at ASGCT 2020

BIOMARKERS

Meaningful Decrease of ~50% in Creatine Kinase Levels in the High Dose Cohort

Cohort	Subject	Day 1, (Pre-Dose)	Day 360	% Change Baseline to D360	% Change Baseline to D360 Mean (± SD)
Control	CT 1*	3,013	5,844	+94%	+17% (± 67.0)
	CT 2	24,875	21,447	-15%	
	CT 3	17,170	13,360	-28%	
Low Dose (5E13 vg/kg)	Pt 1*	4,583	3076	-33%	+166% (± 147%)
	Pt 2	12,385	26,685	+215%	
	Pt 3	6,354	20,200	+318%	
High Dose (2E14 vg/kg)	Pt 4	16,440	7,985	-51%	-55% (± 7.5%)
	Pt 5	12,672	4,291	-66%	
	Pt 6**	10,752	5,447	-49%	

*Patients CT 1 and PT 1 were non-ambulatory (>14 years old) at the time of Baseline measurement.
**Patient 6 was infused after 6 months of follow-up in the delayed treatment cohort.
Pre-dose level was taken on the day of dosing for subjects who received SGT-001 treatment.

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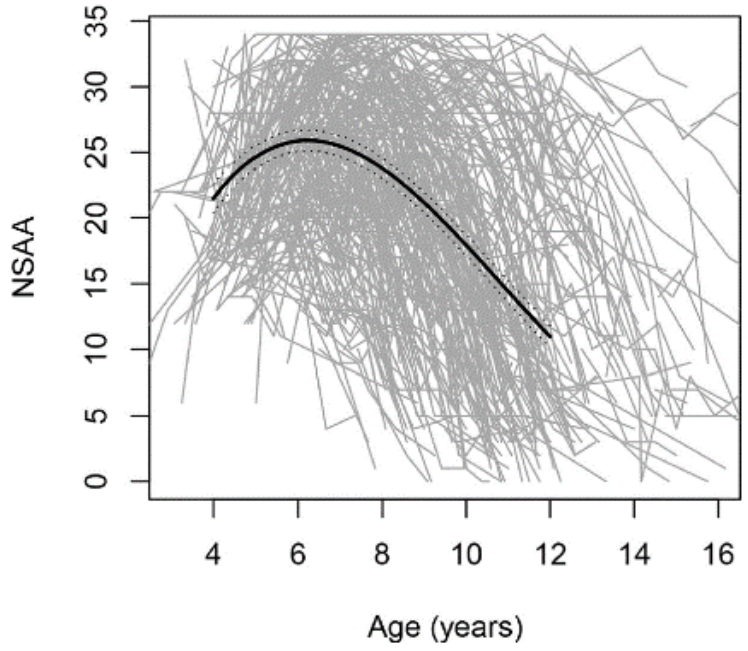
The Natural History of Duchenne Provides Useful Context for Interpreting Changes in NSAA in Response to Treatment

- Consensus that NSAA scores increase up to 6.5 - 7 years of age
- NSAA scores subsequently decline approximately 3.7 points per year after age 7

Natural History		
Age (Years)	**Mean NSAA**	**Mean NSAA Change**
5	25	+2/year up to age 7
7	27.4	
>7	23.7	-3.7/year after age 7

Table data from Ricotti et al., 2015; Figure from Muntoni F, et al., 2019

North Star Ambulatory Assessment (NSAA)



NSAA is a 17-item rating scale used to assess motor function in ambulant Duchenne patients

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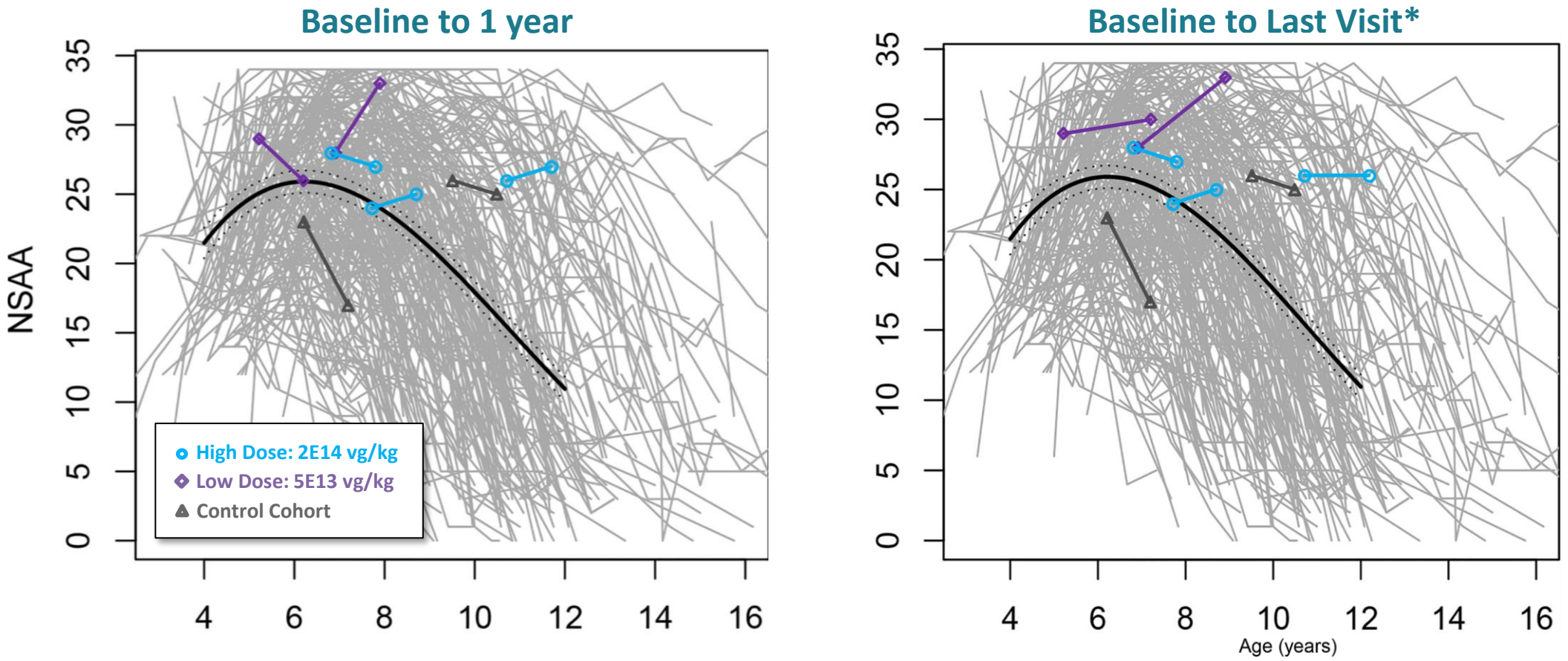
Improvement in NSAA Score Suggests Sustained Benefit in Treated Subjects

Relative to Trajectories Typically Seen in Natural History

NSAA Total Score†: Baseline to 1 Year / Last Visit vs Natural History**



†Subject CT 1 was non ambulant. NSAA was not assessed

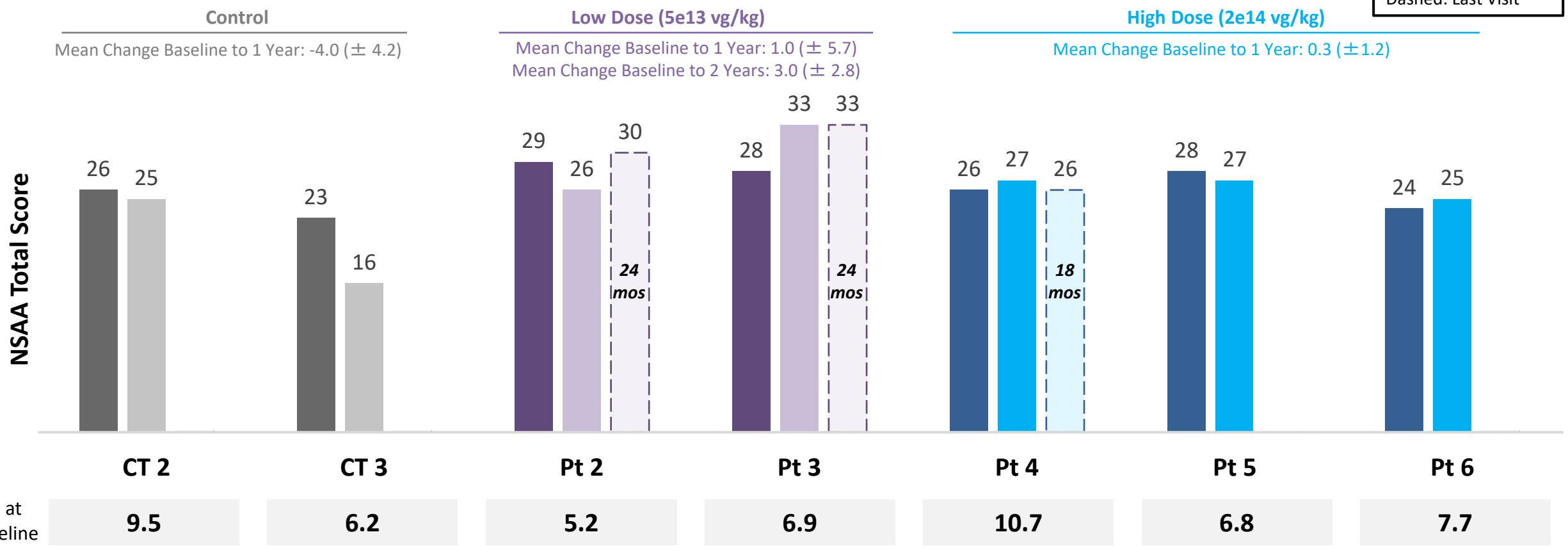
*For Patients 2 and 3 (low dose), last visit was 24 months. For Patient 4 (high dose), last visit was 18 months. For all other patients, last visit was 12 months.

**Muntoni F, et al. (2019)

High Baseline NSAA Observed in Subjects Enrolled in IGNITE-DMD

NSAA Total Score*: Baseline to 1 Year / Last Visit**

Dark: Baseline
Light: 1 Year
Dashed: Last Visit**



	CT 2	CT 3	Pt 2	Pt 3	Pt 4	Pt 5	Pt 6
Age at Baseline	9.5	6.2	5.2	6.9	10.7	6.8	7.7

*Subjects CT 1 and PT 1 were non ambulant. NSAA was not assessed
**For Patients 2 and 3, last visit was 24 months. For Patient 4, last visit was 18 months. For all other patients, last visit was at one year

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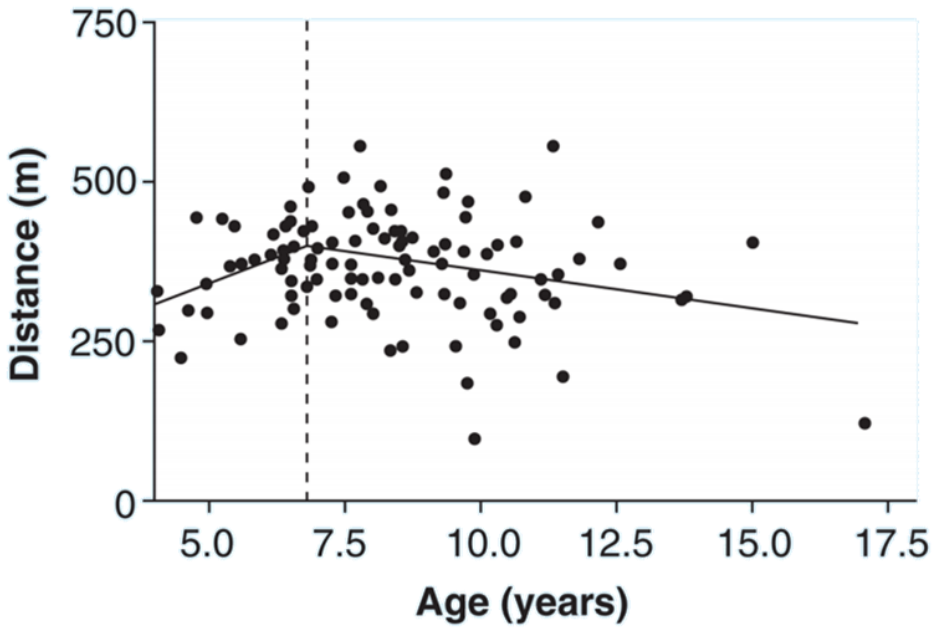
Natural History Reflects a Decline in 6MW Distance of ~35-50m per Year, Beginning Around the Age of 7.

Bushby and Conner. 2011 (n=112)



Arora et al. 2018

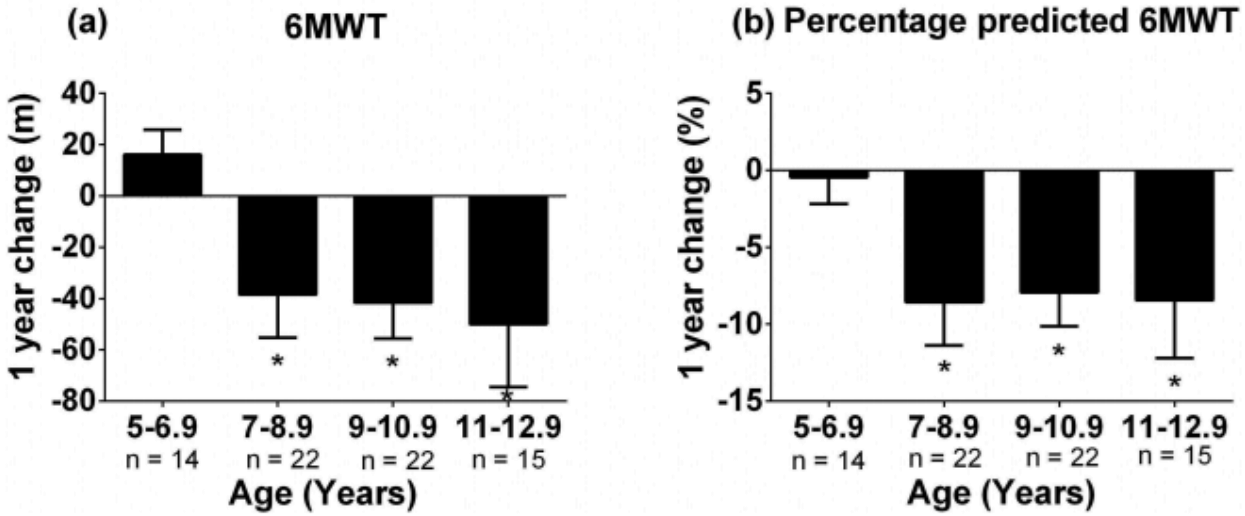


Figure 1. Decline in 6 minute walk test performance over 1 year across ages
A significant decline in 6 minute walk test over 1 year was found for boys >7 years of age for both (a) 6 minute walk test and (b) percentage predicted 6 minute walk test, $p < 0.05$.

These data provide a foundation on which to assess the effect of SGT-001 on 6MW distance among subjects in IGNITE DMD

Figures taken from Bushby and Conner. Clin Investig (Lond). 2011 September
Arora et al. Muscle Nerve. 2018. November.

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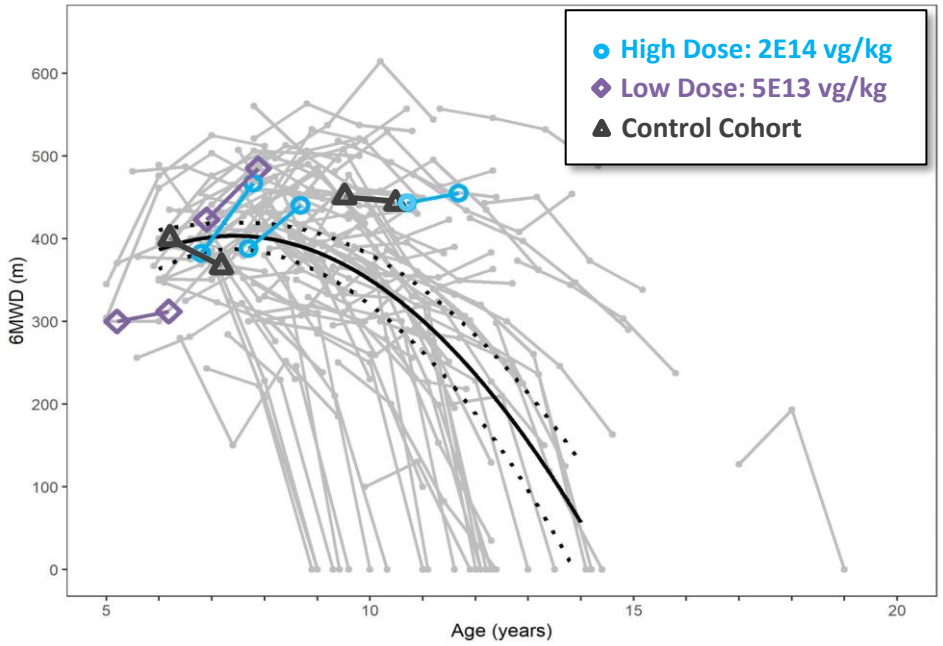
Improvement in 6MWT Distance Among Treated Subjects

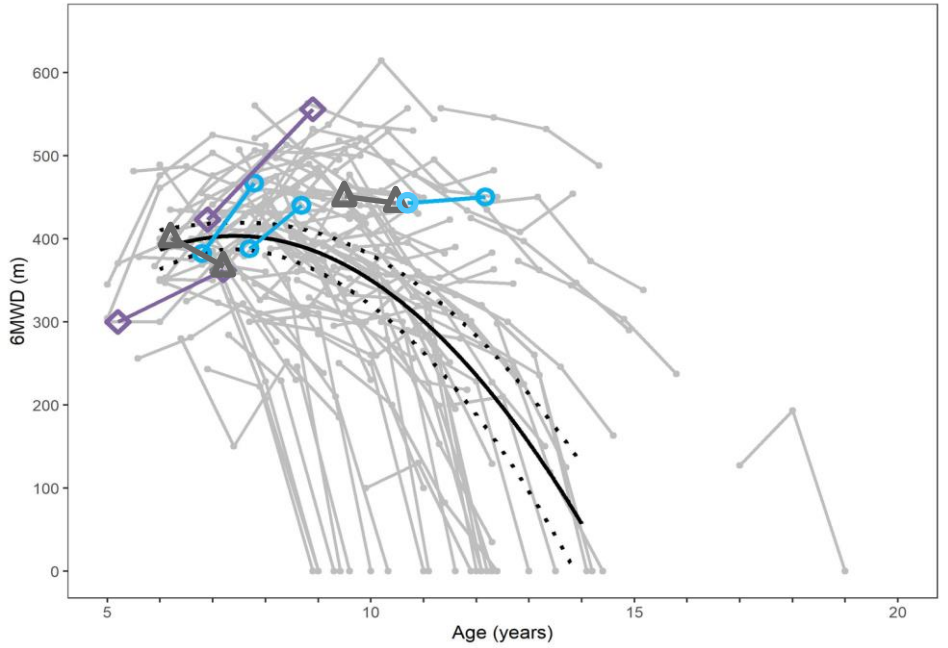
Relative to Trajectories Typically Seen in Natural History

6MWT†: Baseline to 1 Year / Last Visit vs Natural History**

Baseline to 1 year



Baseline to Last Visit*



†Subject CT 1 was non ambulant. 6MWT was not assessed

*For Patients 2 and 3, last visit was 24 months. For Patient 4, last visit was 18 months. For all other patients, last visit was 12 months.

**Muntoni F, et al. (2019)

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Clinically Meaningful Improvements in 6MWT Distances After One Year

Dark: Baseline
Light: 1 Year
Dashed: Last Visit**

6MWT *: Baseline to 1 Year and Last Visit**

Control

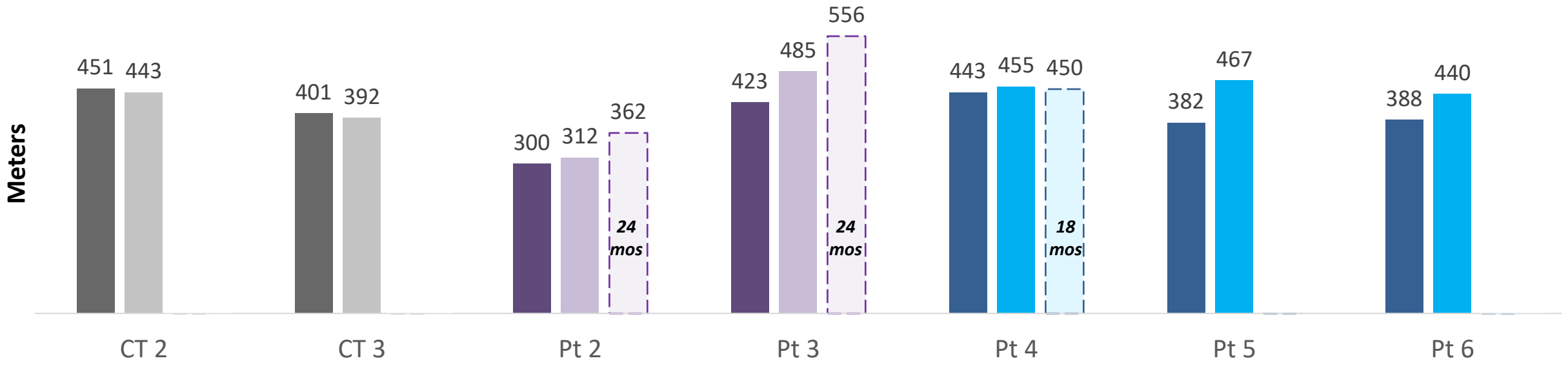
Mean Change Baseline to 1 Year: -8.5 (± 0.7)

Low Dose (5e13 vg/kg)

Mean Change Baseline to 1 Year: 37.0 (± 35)
Mean Change Baseline to 2 Years: 97.5 (± 50.2)

High Dose (2e14 vg/kg)

Mean Change Baseline to 1 Year: 49.7 (± 37)



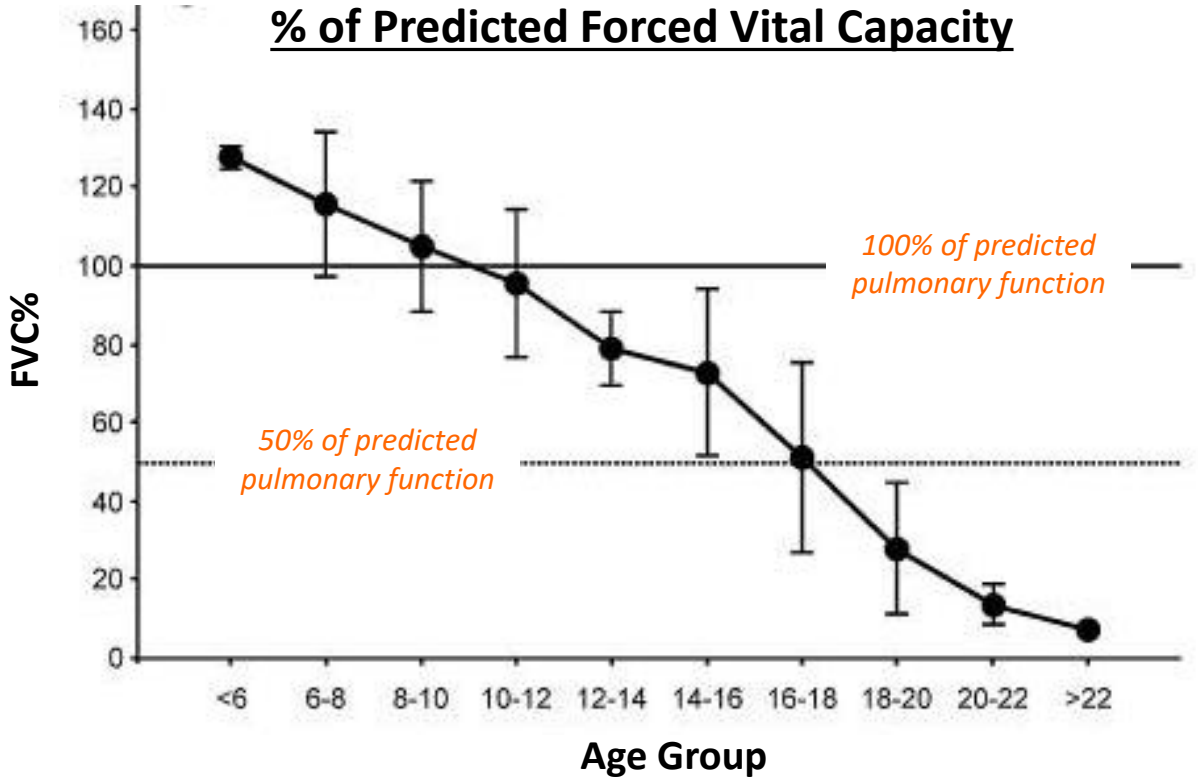
Age at Baseline	CT 2	CT 3	Pt 2	Pt 3	Pt 4	Pt 5	Pt 6
	9.5	6.2	5.2	6.9	10.7	6.8	7.7

Patients across both cohorts showed improvements in 6MWT above the minimally clinical important difference of 30 Meters

*Subjects CT 1 and PT 1 were non ambulant. NSAA was not assessed
**For Patients 2 and 3, last visit was 24 months. For Patient 4, last visit was 18 months. For all other patients, last visit was at one year

The Natural History of Boys With DMD Reveals a Steady Decline in Pulmonary Function, When Normalized for Age and Height

Absolute FVC values progressively increase until mid-teens, but when normalized for age, gender, race and height, boys with DMD exhibit a steady decline from age six onward

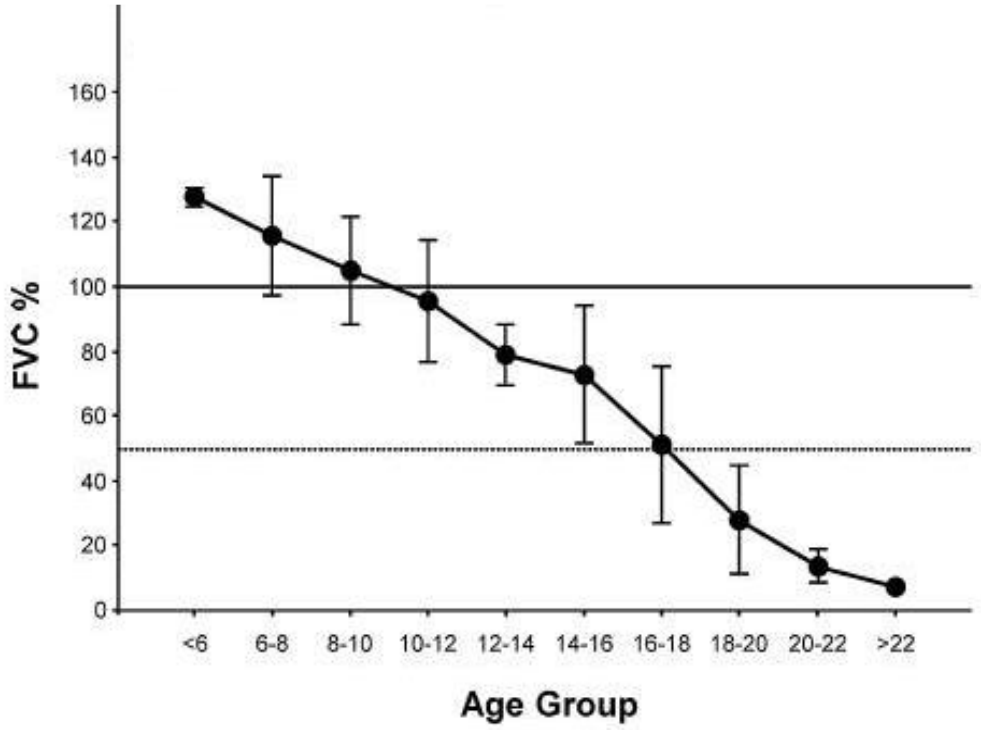


Source: Characterization of Pulmonary Function in Duchenne Muscular Dystrophy. Mayer, et al. Pediatric Pulmonology 50:487–494 (2015)

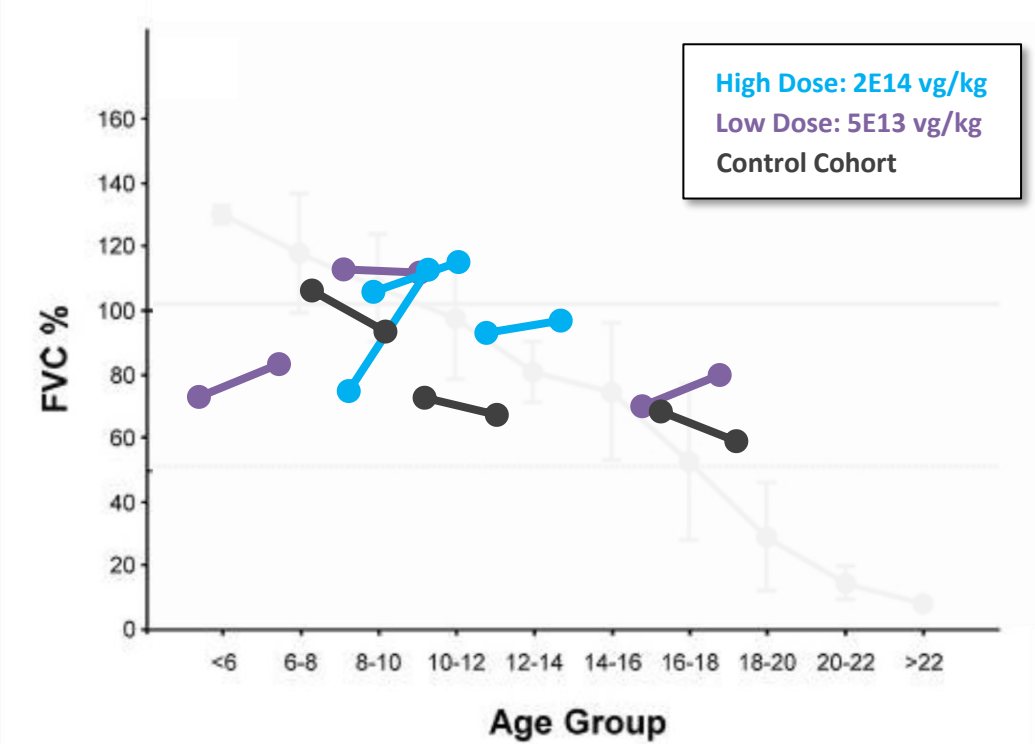
Subjects Treated With SGT-001 Exhibited Improved Pulmonary Function At One Year, When Natural History Would Otherwise Predict Decline

% of Predicted Forced Vital Capacity

Natural History of Pulmonary Function in Boys With DMD.
Mayer, et al. 2015



Pulmonary Function in IGNITE-DMD Subjects:
Baseline vs One Year



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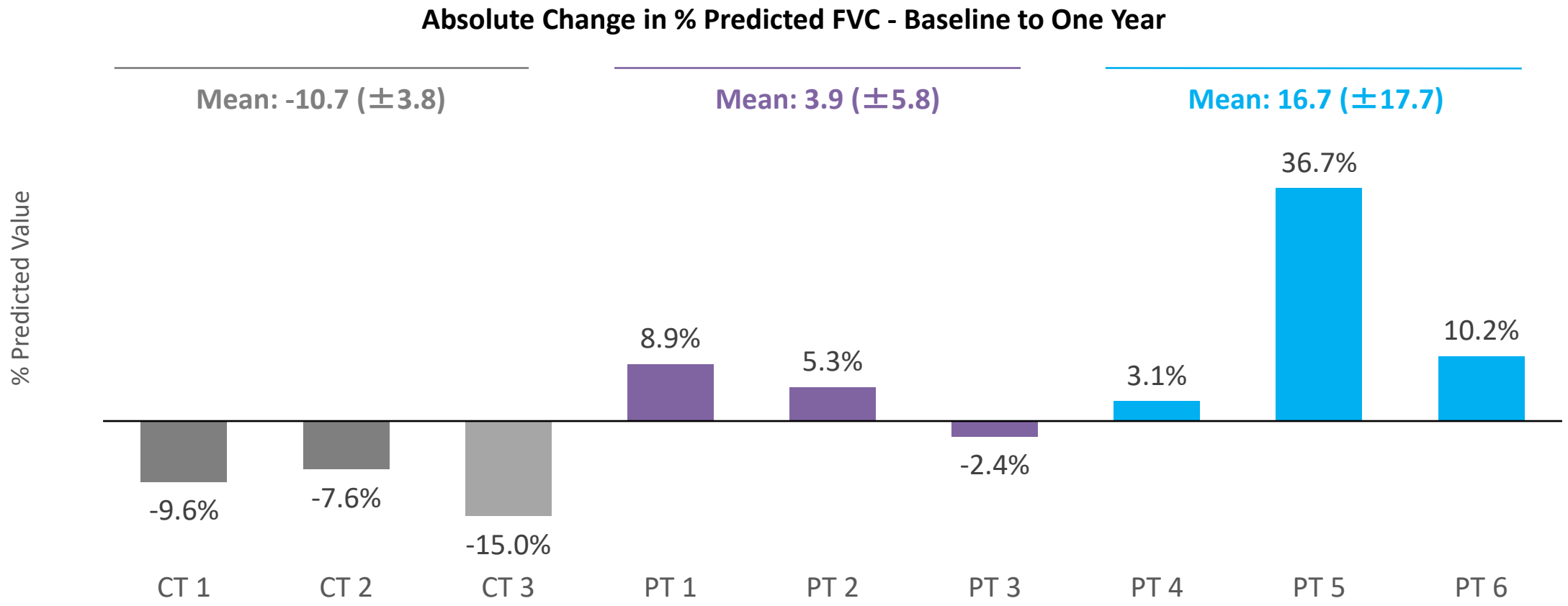
Mean Improvements Were Observed in % Predicted FVC One Year after Treatment with SGT-001



*Values depicted have been normalized to 100% of predicted values based on the Global Thoracic Function Initiative normative equations.

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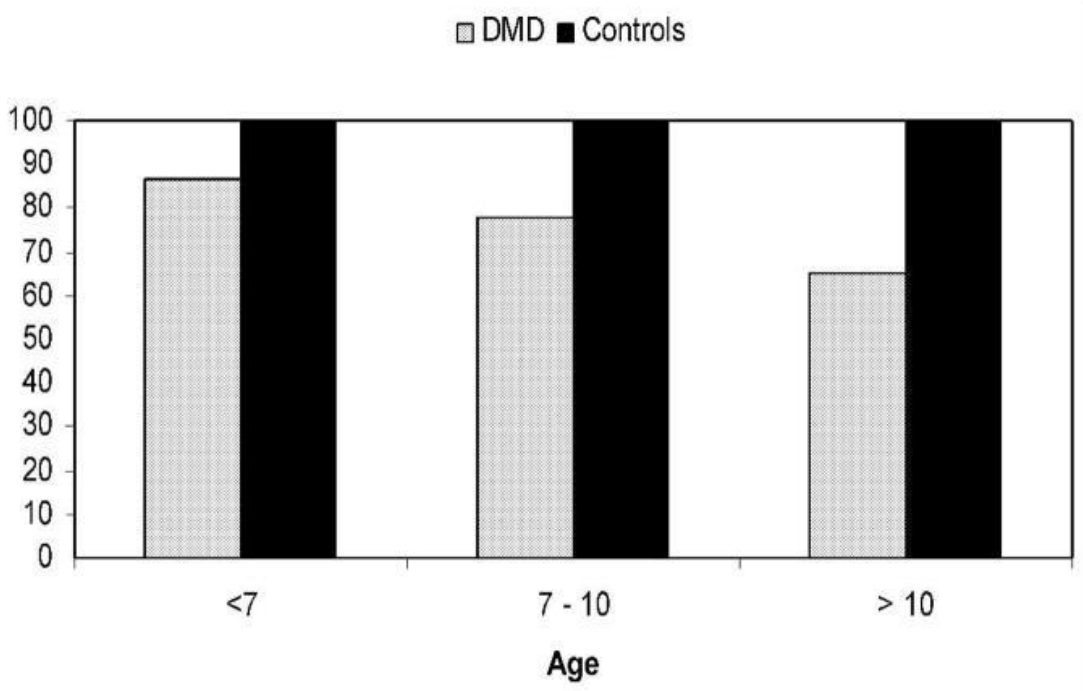
IGNITE DMD Patient Reported Outcomes

Barry Byrne, MD.

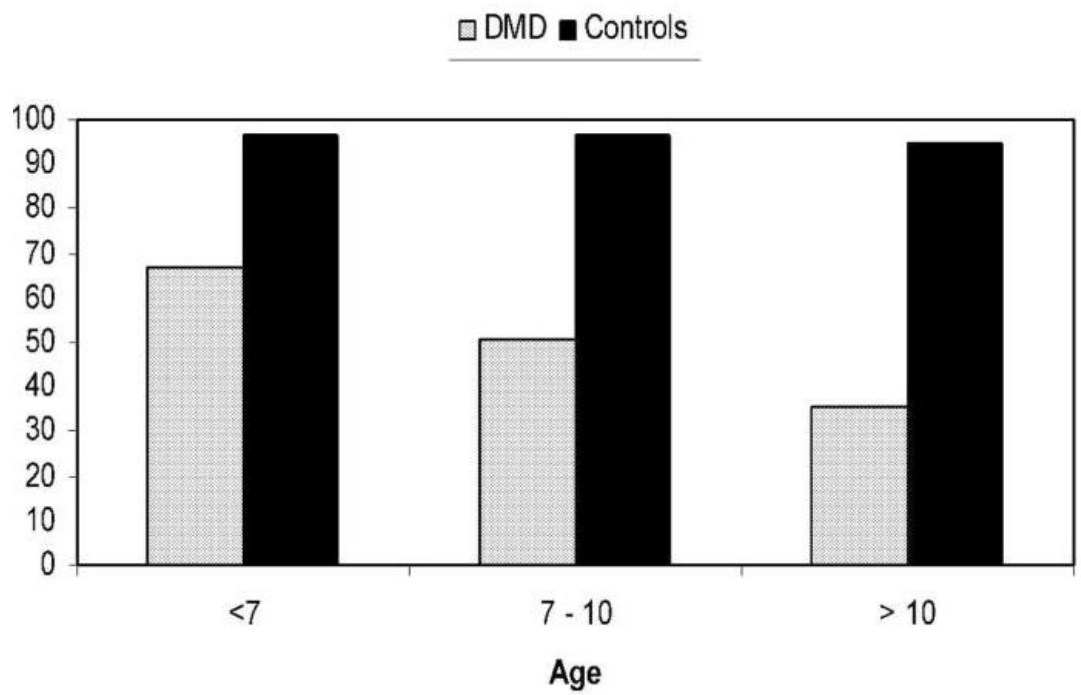
Principal Investigator, IGNITE DMD. Associate Chair of Pediatrics and Director of the Powell Gene Therapy Center at University of Florida

Boys With DMD Experience a Steady Decline in Motor Function vs Healthy Controls As Measured By Key Dimensions of the PODCI

Transfer and Basic Mobility



Sports/Physical Functioning



1: McDonald, CM, et al. Relationship between clinical outcome measures and parent proxy reports of health-related quality of life in ambulatory children with Duchenne muscular dystrophy. J Child Neurol 25:1130-1144, 2010.

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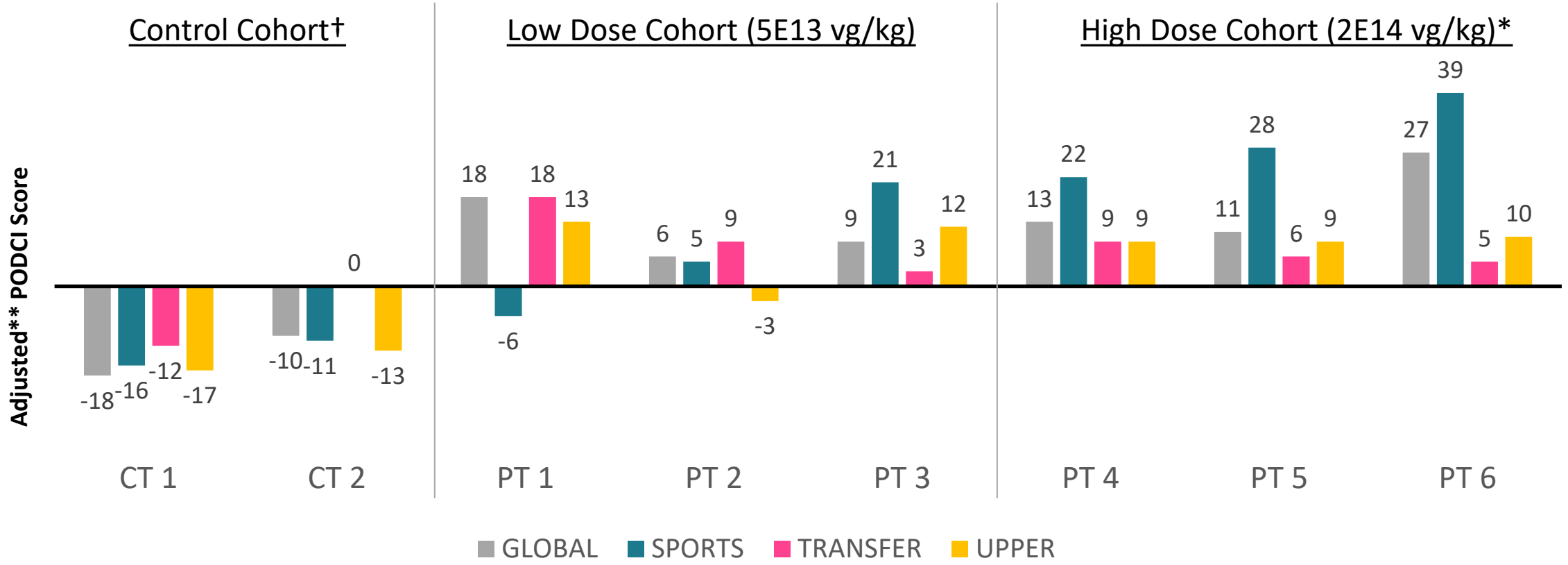
PATIENT REPORTED OUTCOMES

Subjects Treated With SGT-001 Exhibited Meaningful Improvement on the PODCI From Baseline to One Year



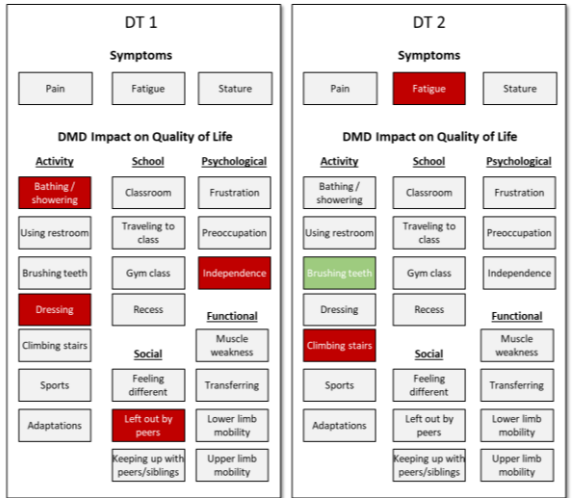
Motor function scores in high dose subjects reflect the benefits exhibited on 6MWT and NSAA observed in these patients.

* Statistically Significant (p=0.05) Improvements Were Observed in the High Dose Cohort in All Domains that Assess Motor Function.
**Standardized scores for each scale are calculated so that a '0' represents a poor outcome/worse health while '100' is the best possible outcome/best health. For the dosing period, Baseline is defined as the last measurement prior to the start of study drug infusion. For the Delayed Treatment period, Baseline is defined as the last measurement on or prior to the Day 1 visit.
† PODCI for Subject CT 3 was not collected at 1 year

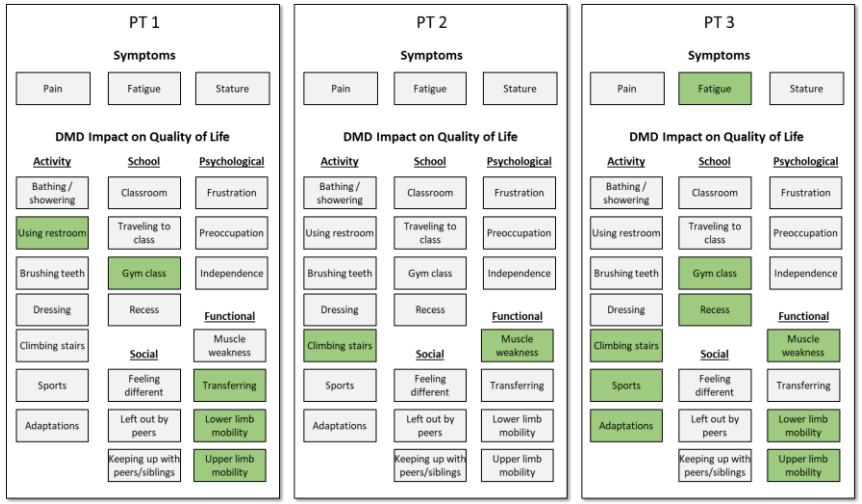
PATIENT REPORTED OUTCOMES

Subjects Treated With SGT-001 Cohort Reported Improvements on a Range of Motor Function Domains

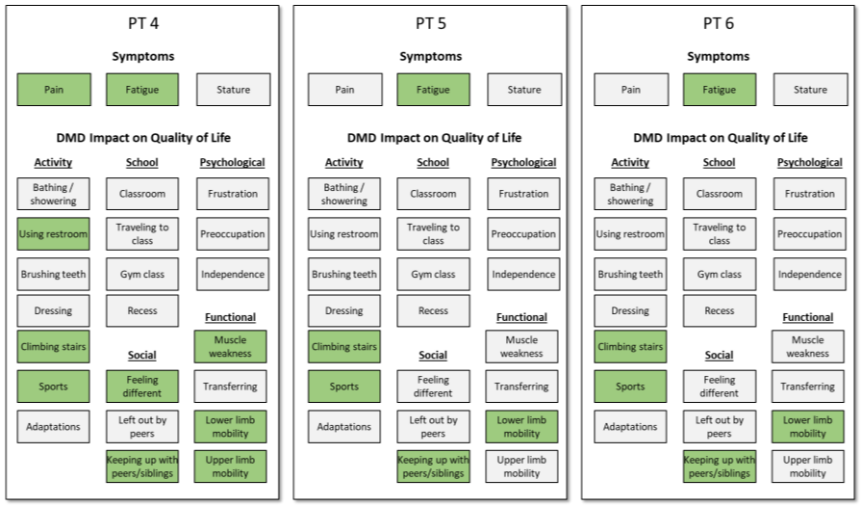
Control*



Low Dose Cohort (5e13 vg/kg)



High Dose Cohort (2e14 vg/kg)



Consistent improvement in motor function domains across all three subjects in the high dose cohort

- Fatigue
- Climbing stairs
- Sports
- Lower limb mobility
- Keeping up with peers/siblings

*Modus Outcomes were not collected for Subject CT 3 at 1 year

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Updates to SGT-001: Clinical Protocol Amendments and 2nd Generation Manufacturing

Joel Schneider, PhD, *Chief Operating Officer*

Clinical Mitigation Strategy and 2nd Generation Manufacturing Process

Clinical Protocol Amendments Made in Consult with FDA and Experts in Immunology and Complement Biology

Designed to Inhibit Complement Activation

Pretreatment with eculizumab and C1 esterase inhibitor

Dampening Immune Response

Transient increase in steroid regimen from 1 mg/kg to 2 mg/kg

Reduce Total Viral Load (TVL)

18 kg weight limitation on next two patients*

2nd generation manufacturing achieved ~50% reduction in total viral load

SGT-001 manufactured under 2nd generation process use for Patient 7 and all subsequent subjects in IGNITE DMD

*Resume dosing in smaller patients to decrease TVL; adjust weight upwards as appropriate

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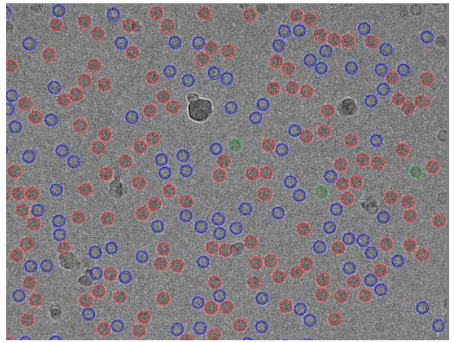
Clinical Mitigation Strategy and 2nd Generation Manufacturing Process

Improvement from 50% to 90% full capsids in final drug product

% of Full vs Empty Capsids

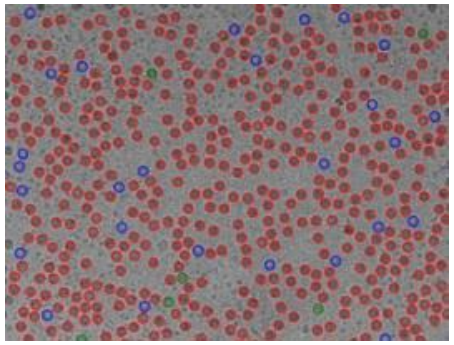
50% full



1st Gen AAV Process

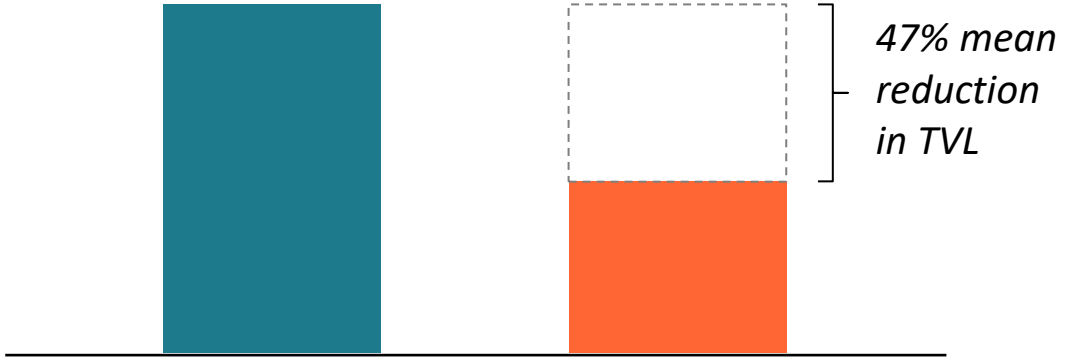
→

>90% full



2nd Gen AAV Process
(NEW)

Total Viral Load at 2e14 vg/kg



1st Gen AAV Process

2nd Gen AAV Process
(NEW)

Reduced total viral load expected to improve dosing safety of SGT-001

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Patient 7 Dosed With SGT-001 Under The Amended Clinical Protocol With Supply From The 2nd Generation Manufacturing Process - No SAEs Reported

Criteria Defined A Priori to Determine Effectiveness of Risk Mitigation:

- Absence of SAEs
- Absence of complement-mediated AEs
- Substantial reduction in laboratory measures of complement activation compared with previously dosed patients

Screening for additional subjects in 2021 ongoing

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Closing Remarks

Ilan Ganot, *Co-founder, President, and CEO*

Key Takeaways From Interim Analysis of IGNITE DMD

Encouraging evidence of functional benefit after 1 year of treatment vs natural history

- ✓ 6 Minute Walk Test (6MWT);
- ✓ North Star Ambulatory Assessment Total Score (NSAA)
- ✓ Forced Vital Capacity (FVC) normalized for age, height, and weight

Meaningful improvement in patient reported outcomes that assess motor function and fatigue

- ✓ Pediatric Outcomes Data Collection Instrument (PODCI)
- ✓ Modus Outcomes results

Interim results potentially supportive of the role of neuronal nitric oxide synthase (nNOS)

Totality of data supports continued dosing of IGNITE DMD at 2e14 vg/kg dose

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Closing Remarks

		CK (U/L)	NSAA	6MWT (m)	FVC%	PODCI – Sports	PODCI – Global
Control	CT 1 *15.3 yrs*	+2,831	n/a	n/a	-9.6%	-16	-18
	CT 2 *9.5 yrs*	-3,428	-1	-8	-7.6%	-11	-10
	CT 3 *6.2 yrs*	-3,810	-7	-9	-15.0%	n/a	n/a
5E13 vg/kg	Pt 1 *14.4 yrs*	-1,507	n/a	n/a	+8.9%	-6	+18
	PT 2 *5.2 yrs*	+14,300	-3	+12.0	+5.3%	+5	+6
	PT 3 *6.9*	+13,846	+5	+62.0	-2.4%	+21	+9
2E14 vg/kg	PT 4 *10.7yrs*	-8,455	+1	+12.0	+3.1%	+22	+13
	PT 5 *6.8 yrs*	-8,381	-1	+85.0	+36.7%	+28	+11
	PT 6 *7.7*	-5,305	+1	+52.0	+10.2%	+39	+27

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Thank You

Questions & Answers