Solid Biosciences

Q1 2021 Business Update and Financial Results

May 14, 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, the Company’s SGT-003 pipeline program 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, 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 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.

2021 Priorities and Anticipated Milestones

- ✓ **Resume dosing patients in IGNITE DMD** (Q1 2021)
- ✓ **Present 12-month safety & efficacy for patients 1-6** (Q1 2021)
- ✓ **Further pipeline expansion**

- **Present 90-day biopsy data for additional patients dosed in IGNITE DMD** (2H 2021)
- **Advance towards commercial readiness**
- **Prepare for registration study**

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SGT-001 Clinical Program

IGNITE DMD Dosing Update

Patient 8 Post Dosing Clinical Course

- Patient 8 experienced an inflammatory response, which was classified as a serious adverse event (SAE) and considered by the investigator to be drug-related.
- Components of this SAE were similar to inflammatory responses seen in other patients but less severe than Patient 6
- As of his 30-day visit, most laboratory values had returned to normal or continue to trend towards normal
- Data on this SAE have been shared with FDA and DSMB
- Internal teams and external experts are doing an extensive analysis of the SAE to gain insight into its cause and determine potential steps to further enhance patient safety

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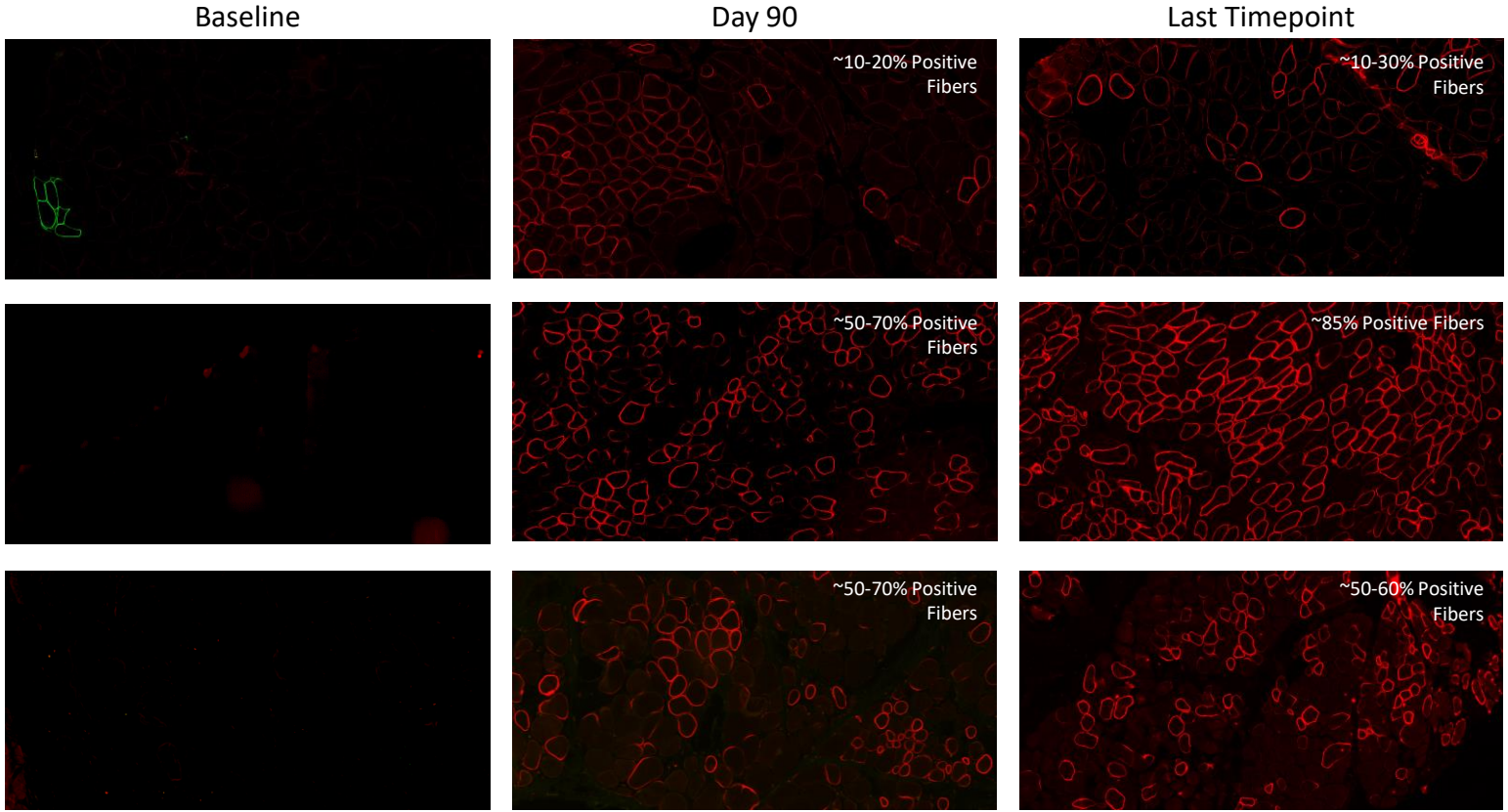
SGT-001 Update

Long-Term Protein Expression Data

Functional Data Summary

Durable, Muscle-Wide Microdystrophin Expression in All Patients Dosed at 2E14 vg/kg



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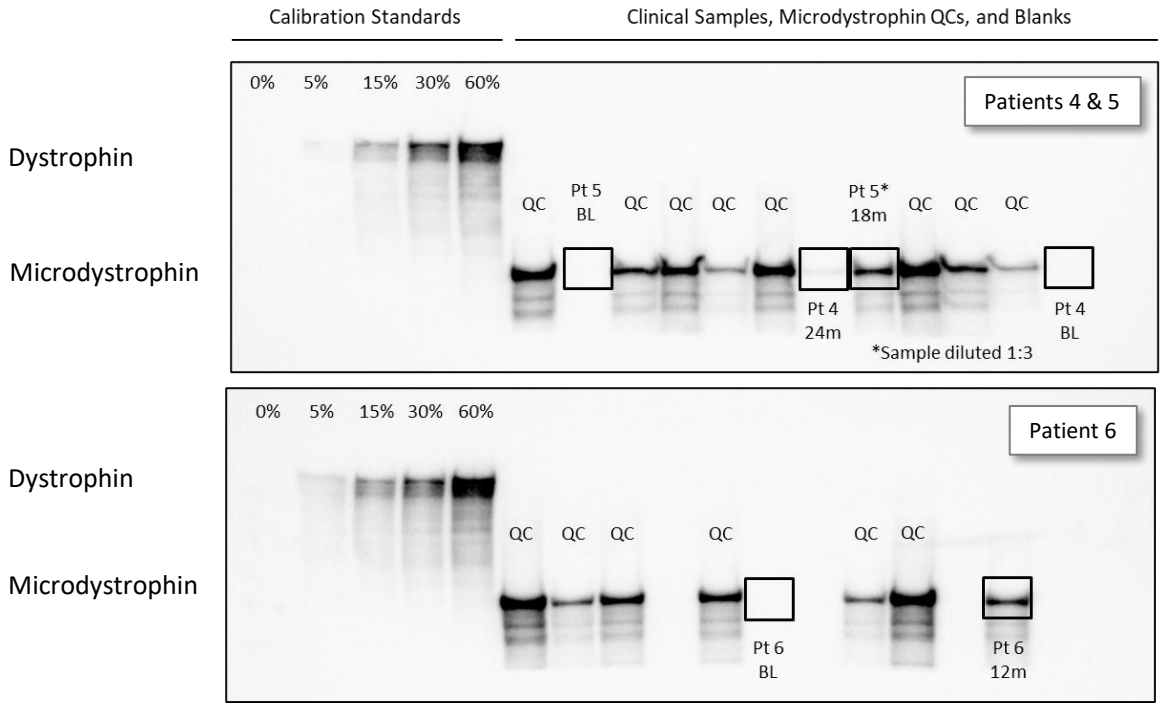
Persistent Microdystrophin Expression Observed in Long-Term Biopsies

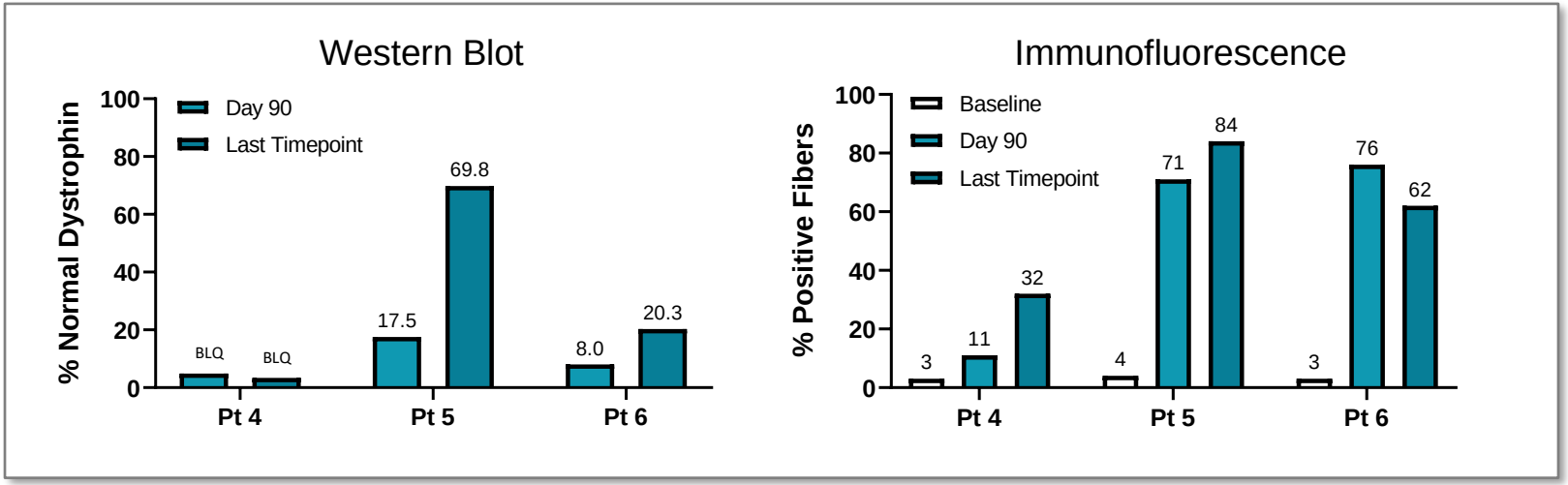
Quantitation of Microdystrophin Expression via Western Blot



Comparison of Microdystrophin Expression

	% Normal Dystrophin	
	Day 90	Last Timepoint
Pt 4	BLQ	BLQ (24 months)
Pt 5	17.5%	69.8% (18 months)
Pt 6	8.0%	20.3% (12 months)

Sustained Microdystrophin Expression at ≥12 months



Patient	% Normal Dystrophin (WB)		% Microdystrophin Positive Fibers (IF)	
	Day 90	Last Timepoint	Day 90	Last Timepoint
Pt 4	BLQ	BLQ	11%	32%
Pt 5	17.5%	69.8%	71%	84%
Pt 6	8.0%	20.3%	76%	62%

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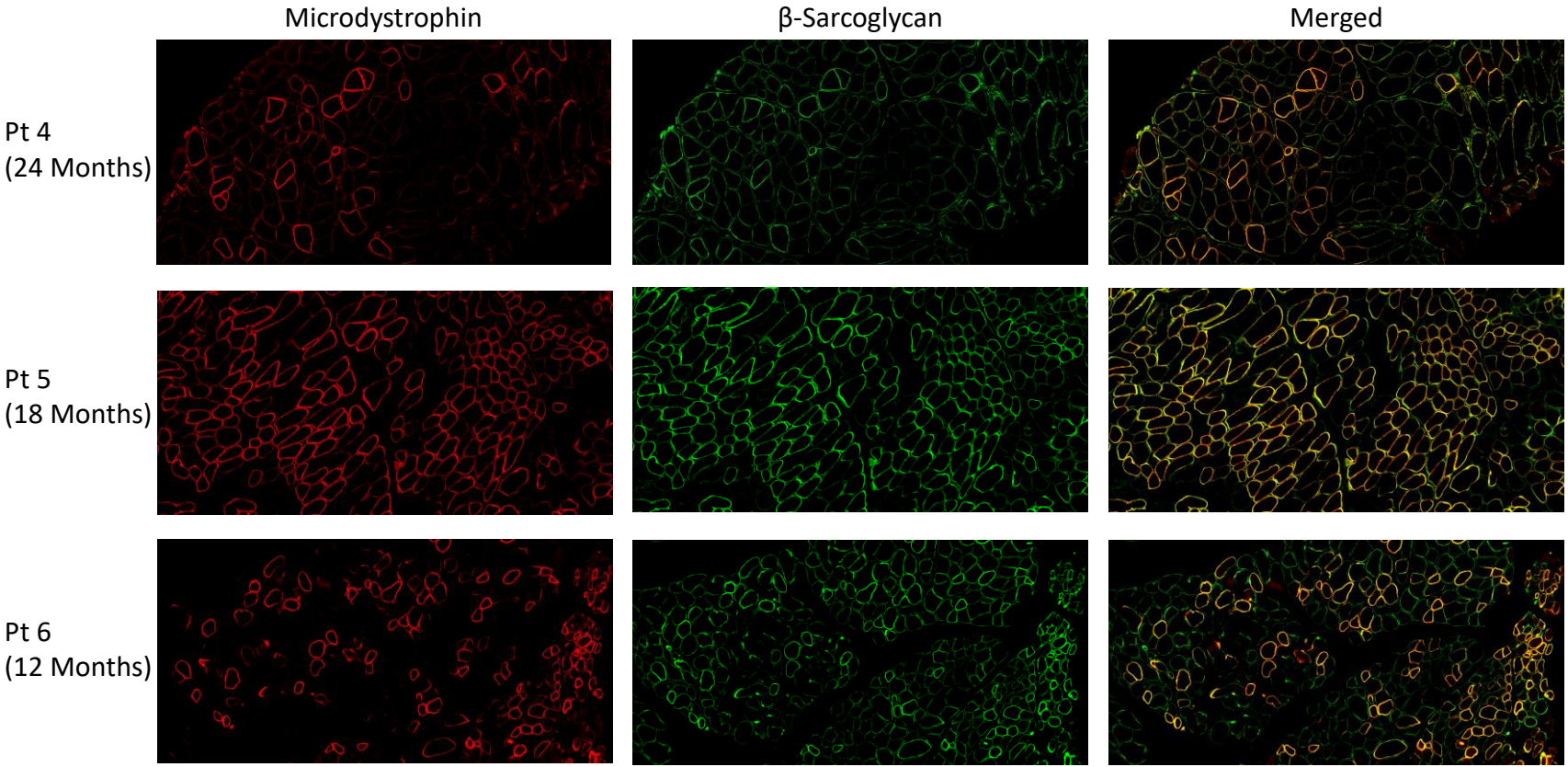
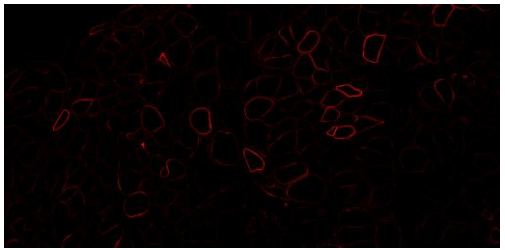
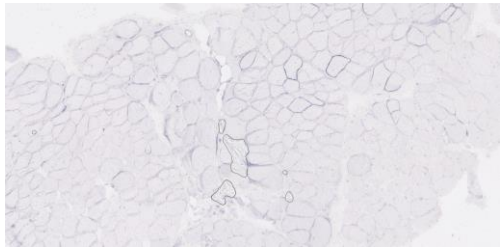
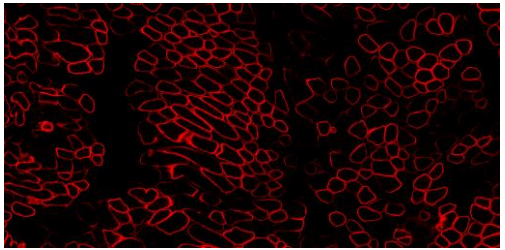
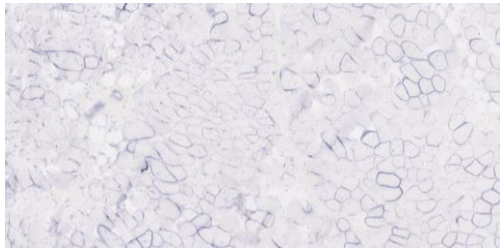
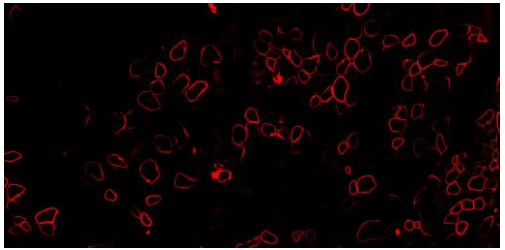
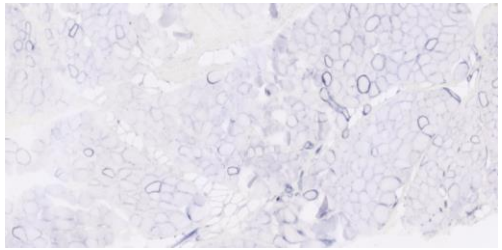
Microdystrophin Function: Restoration of β-Sarcoglycan to the Sarcolemma



Microdystrophin Function: Restoration of Enzymatically Active nNOS to the Sarcolemma

	Microdystrophin	nNOS Localization / Activity
Pt 4 (24 Months)		
Pt 5 (18 Months)		
Pt 6 (12 Months)		

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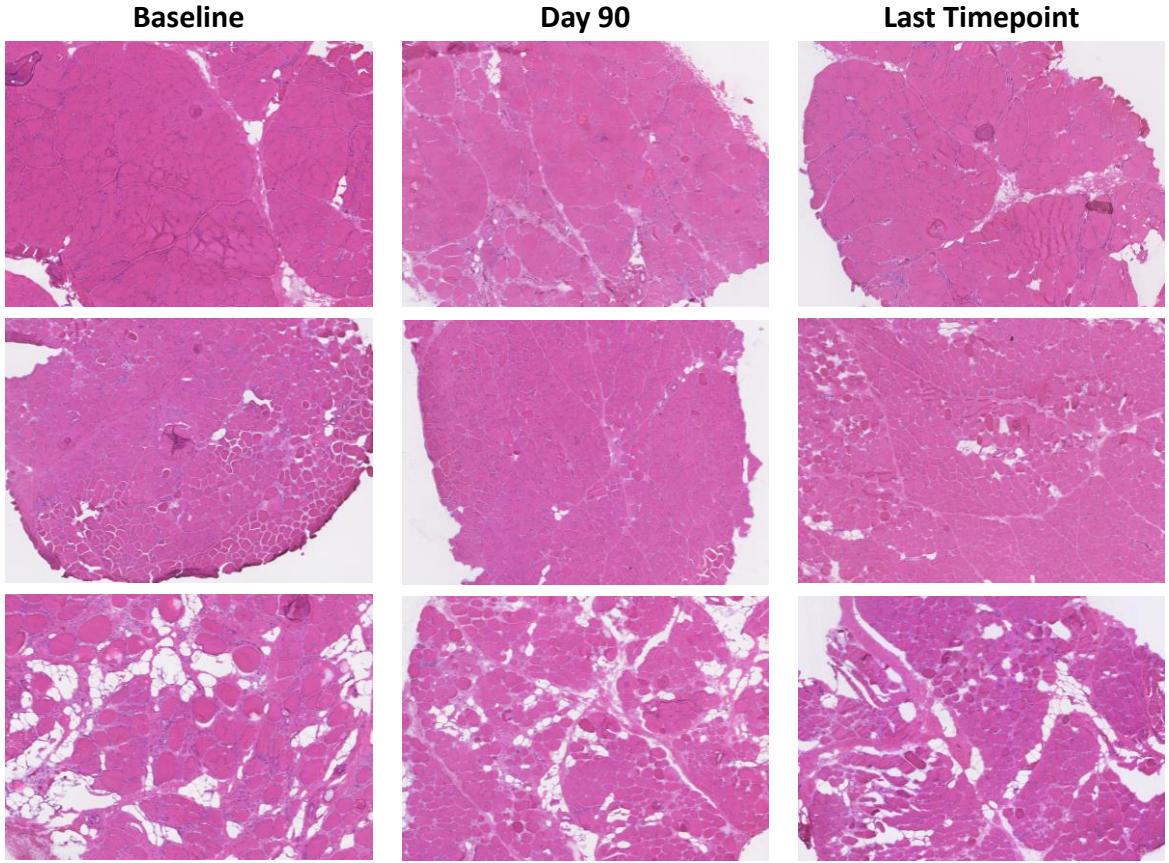
Limited Dystrophic Pathology Progression Observed in Long-Term Biopsies



24 months (Age 12.7 yrs)

Very mild active dystrophic pathology

18 months (Age 8.3 yrs)

No active dystrophic pathology

12 months (Age 8.7 yrs)

Very mild active dystrophic pathology

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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 *10.7yrs*	-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 yrs*	-5,305	+1	+52.0	+10.2%	+39	+27

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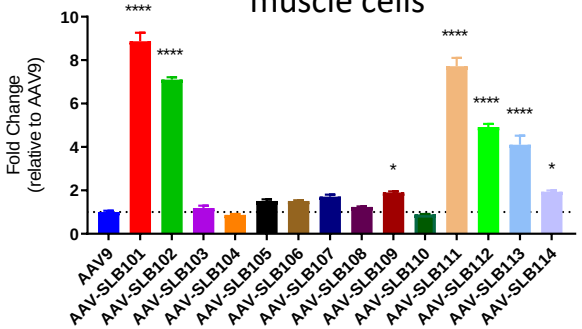
Pipeline Update

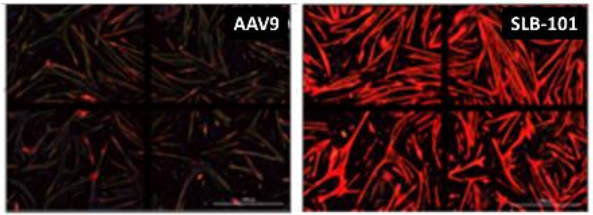
SGT-003

Ultragenyx Collaboration

Development of Next Generation Capsids Designed to Enhance Muscle Transduction Efficiency

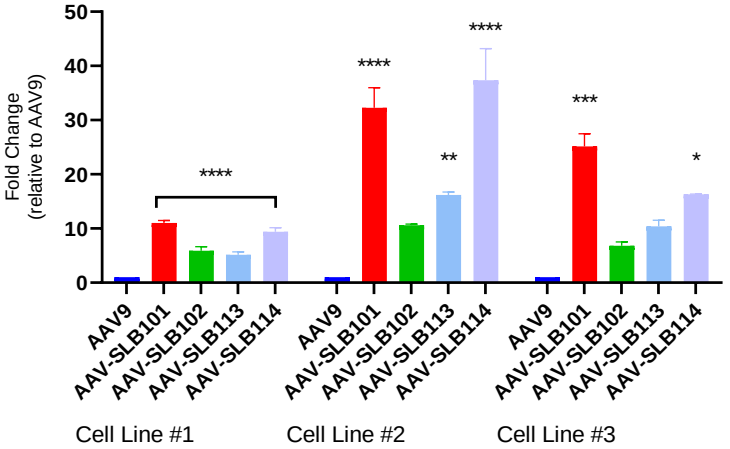
Initial screening of novel AAV capsids via measurement of Microdystrophin expression in C2C12 muscle cells





SLB-101 shows ~9-fold increased expression vs AAV9

Selected candidates were further screened for Microdystrophin expression in human DMD muscle cell lines

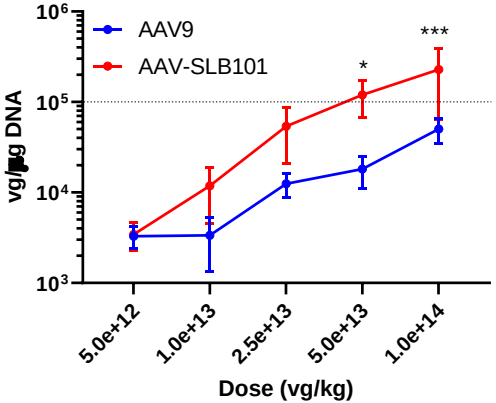


10-30X increase in Microdystrophin expression in three human DMD cell lines with SLB-101 vs AAV9

*Poster presentations at ASGCT 23rd and ASGCT 24th Annual Meetings

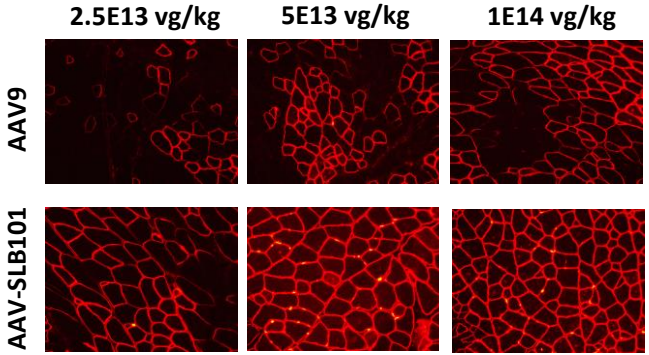
SLB-101 capsid selected for advancement - exhibits increased muscle tropism in mdx mice

Biodistribution (vg/µg gDNA) in Skeletal Muscle



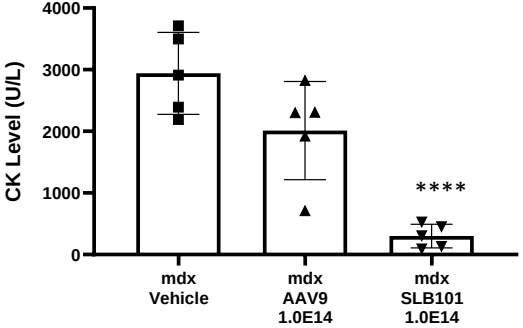
3-7-fold increase in biodistribution in skeletal and cardiac muscle with decrease seen in liver

Microdystrophin Expression in Skeletal Muscle



Up to 3X dose-dependent improvement in Microdystrophin expression compared to AAV9 in vivo

Creatine Kinase Levels in Serum



Significant reduction in serum CK activity, a biomarker of muscle damage compared to AAV9

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*Poster presentation at ASGCT 24th Annual Meeting

Collaboration to Advance Next Generation DMD Constructs

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- Proprietary nNOS-binding form of microdystrophin
- World class expertise in Duchenne and muscle biology

ultragenyx pharmaceutical

- HeLa PCL Platform: Commercial-grade 2,000L manufacturing capability
- AAV8 Variant with favorable immune profile

- Collaboration is effectively leveraging each side's expertise and resources
- Ultragenyx is leading efforts around vector construction, optimization and creation of HeLa producer cell line
- *In vitro* and *in vivo* screening of novel vectors expedited by routing expression analytics through Solid's research team and leveraging our established assays
- Additional update expected by the end of 2021

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Increased Financial Strength

$143.8 Million

OF CAPITAL RAISED IN Q1 2021

$268.5M

Cash and cash equivalents as of 3/31/21

Q4 2022

Current cash and cash equivalents expected to funder operating expenses into Q4 2022

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Thank You

Q&A