



Solid Biosciences

Corporate Overview

November 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 predictive 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.



Solid's Mission

Solid Biosciences is a life science company focused on developing transformative treatments to improve the lives of patients living with Duchenne muscular dystrophy. Disease-focused and founded by a family directly impacted, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation with our gene therapy candidate, SGT-001.

An Innovator in Gene Therapy, Solid Bio is Developing Promising Approaches to bring Meaningful Benefits to Patients



Patient Centric

Clinical studies are powered by input from the patient community and designed to gain meaningful insights in order to best understand all facets of the disease



Collaborative

Solutions influenced by the convergence of ideas and knowledge through collaborations with industry, academia, government and community



Center of Excellence

Expertise in muscle biology, gene therapy, clinical, regulatory and patient advocacy allows the Solid team to intuitively develop treatments for musculoskeletal diseases






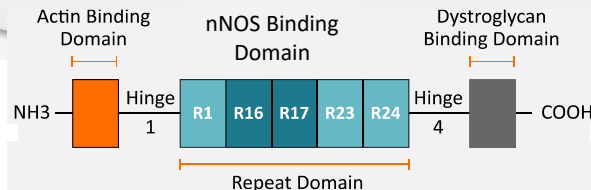
Positioned for Success

Financial runway enables Solid to advance key strategic priorities

Recent data presented on lead program SGT-001 demonstrate potential long-term benefit and durable protein expression. Focus on advancing clinical and pipeline development.

Duchenne Muscular Dystrophy Research & Development Programs

 SGT-001	 SGT-003	 UX-810
<ul style="list-style-type: none"> Investigational gene transfer that enables the systemic delivery of a smaller, but functional version of the dystrophin gene, called microdystrophin, to muscles. SGT-001 is currently being investigated in our Phase I/II IGNITE-DMD clinical trial. 	<ul style="list-style-type: none"> Pre-clinical program incorporating the nNOS binding domain-containing microdystrophin construct. Incorporates a next-generation, rationally designed capsid that has improved efficiency and more targeted transduction of muscle cells. 	<ul style="list-style-type: none"> Collaboration between Solid Biosciences and Ultragenyx to advance next generation DMD constructs. Construct is comprised of Solid's proprietary nNOS-binding form of microdystrophin and Ultragenyx's HeLa PCL manufacturing platform for use with AAV8 and variants thereof.





Corrective Therapies

Innovation in Gene Transfer



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



Capsid



Skeletal and cardiac transduction

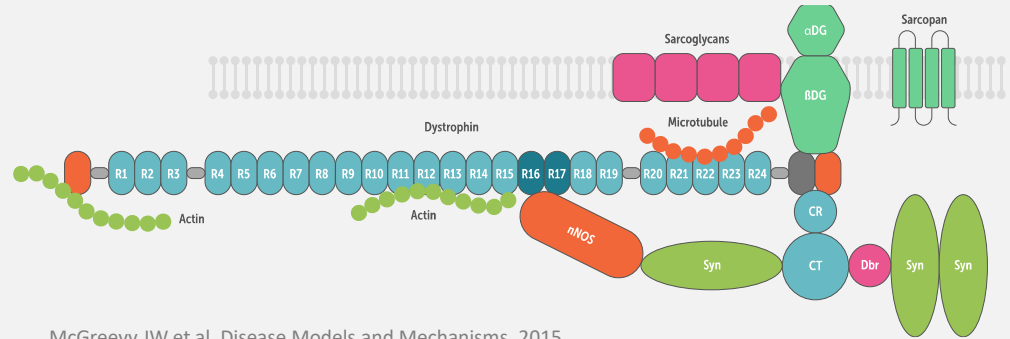


AAV9

SGT-001: Differentiated Microdystrophin Biology

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)

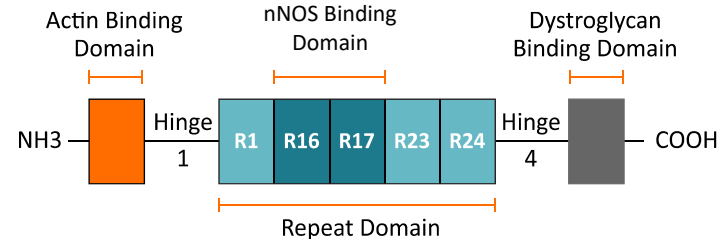


McGreevy JW et al. Disease Models and Mechanisms. 2015

SGT-001 Microdystrophin

- Microdystrophin is a rationally designed recombinant protein
- 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: Retains key dystrophin protein functional domains



*nNOS: neuronal nitric oxide synthase



SGT-001 Clinical Program

IGNITE DMD

Long-Term Protein Expression Data

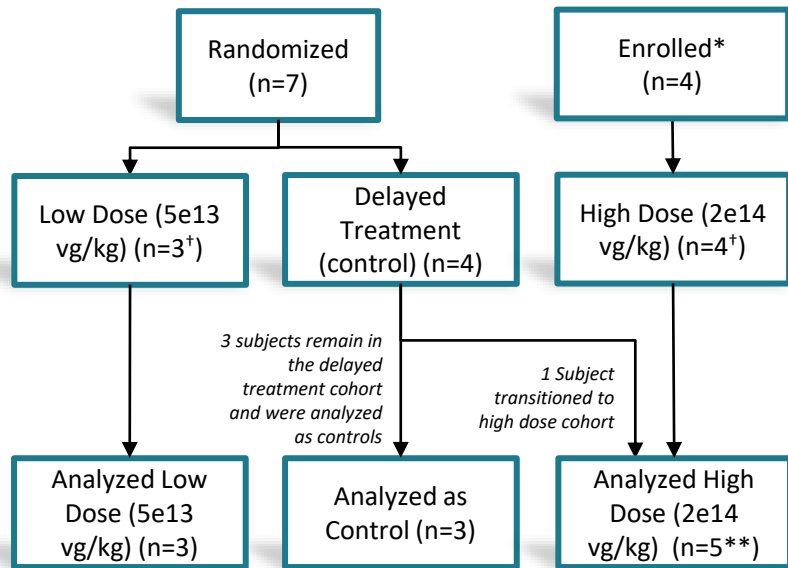
Long-Term Functional Outcomes Data

Pulmonary Function Outcomes Data



IGNITE-DMD Study Design: *Two Dose Levels Initially Assessed; 2e14 vg/kg Selected*

Interim Analysis of Subjects in IGNITE DMD*



*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

**Patients 7 & 8 dosed under new clinical mitigation strategy with improved manufacturing process; Patient 8 experienced an expected SAE which has since fully resolved

Inclusion Criteria Summary

- 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
- Stable cardiac and pulmonary function
- Stable daily dose of corticosteroids > 12 weeks
- For more information, please visit [clinicaltrials.gov NCT03368742](https://clinicaltrials.gov/NCT03368742)

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

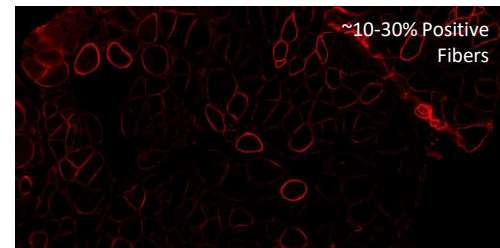
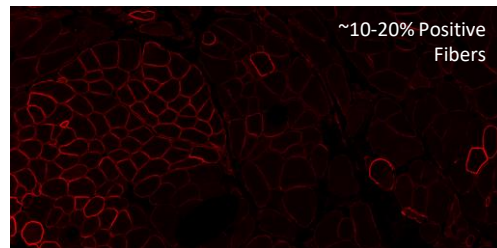
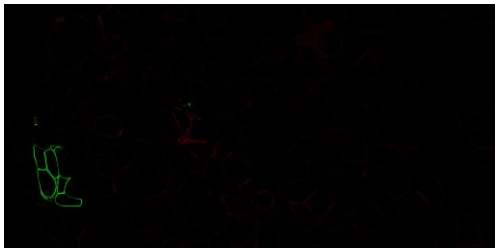
Muscle-Wide Microdystrophin Expression in All Patients Dosed at 2E14 vg/kg 12-24 Months After SGT-001 Administration

Baseline

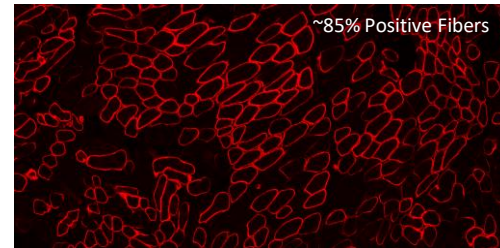
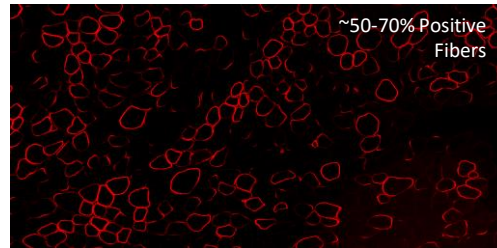
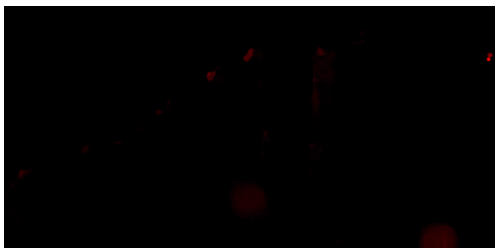
Day 90

Last Timepoint

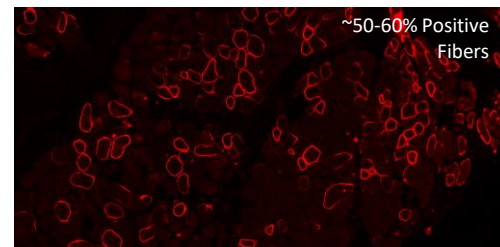
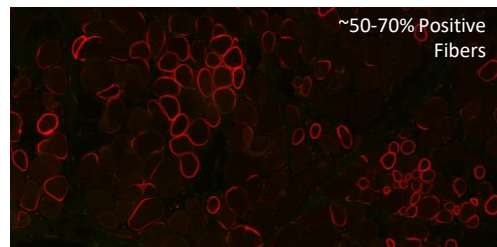
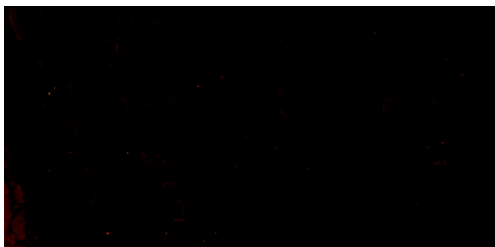
Pt 4: 24 months
(Age at baseline:
10.7 years)



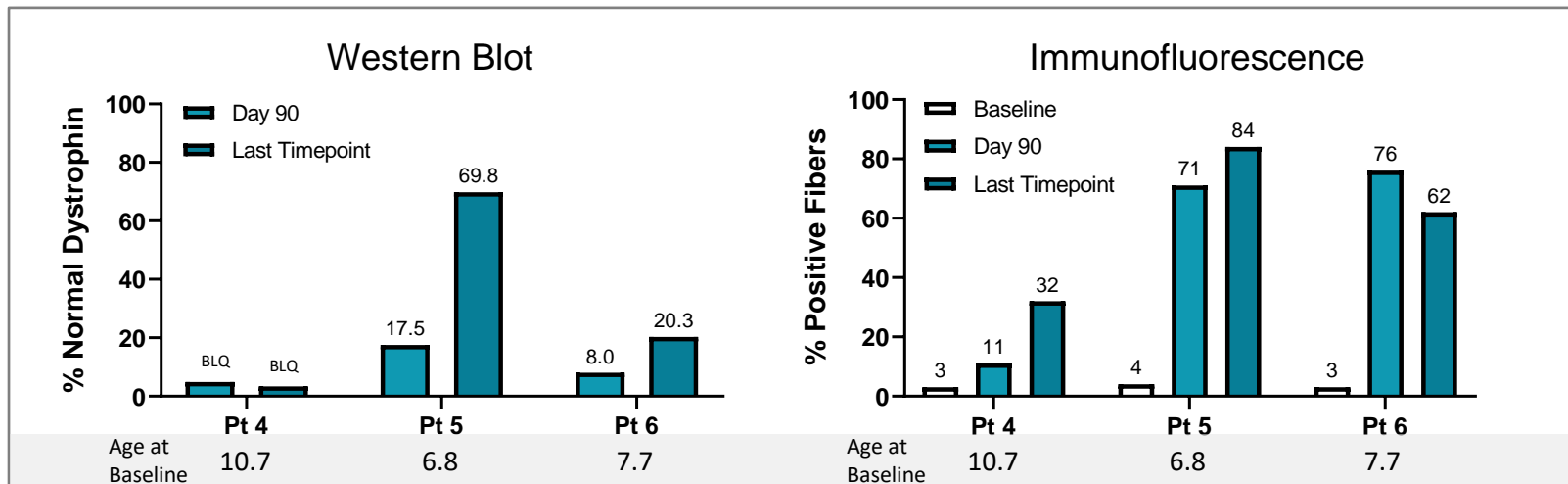
Pt 5: 18 months
(Age at baseline:
6.8 years)



Pt 6: 12 months
(Age at baseline:
7.7 years)



Sustained Microdystrophin Expression at ≥ 12 months



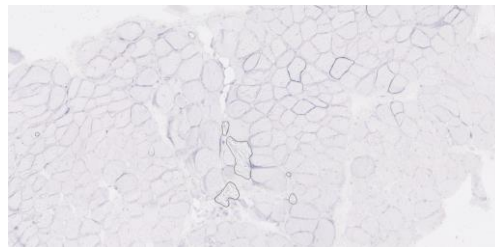
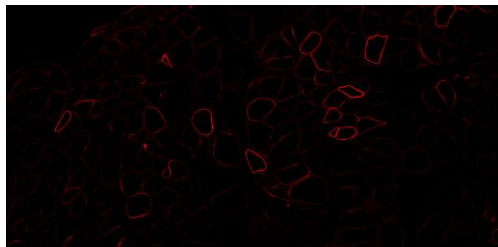
Patient	% Normal Dystrophin (WB)		% Microdystrophin Positive Fibers (IF)	
	Day 90	Last Timepoint	Day 90	Last Timepoint
Pt 4	BLQ	BLQ	11%	32%
Pt 5	17.5%	69.8%	71%	84%
Pt 6	8.0%	20.3%	76%	62%

Microdystrophin Function: Restoration of Nitric Oxide Production

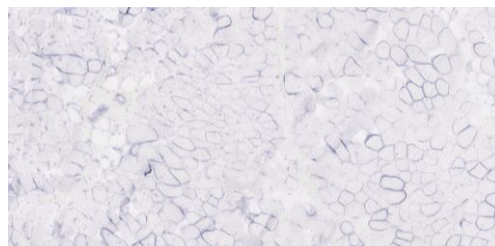
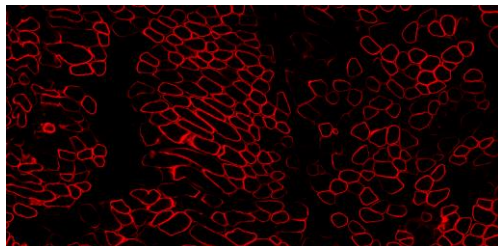
Microdystrophin

nNOS Activity

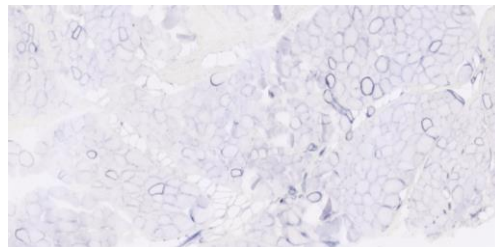
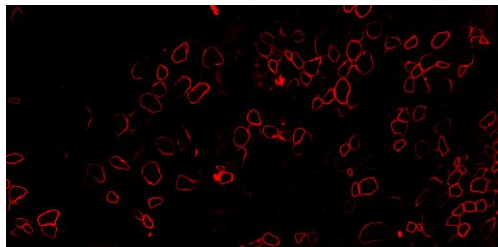
Pt 4: 24 months
(Age at baseline:
10.7 years)



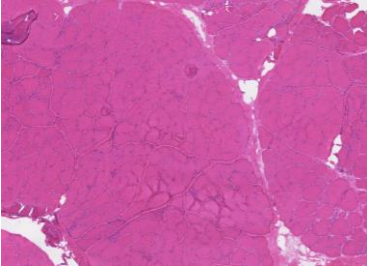
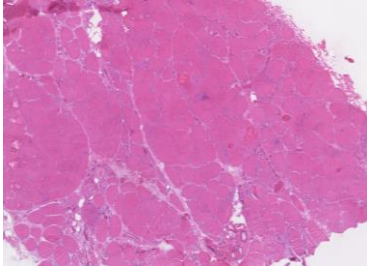
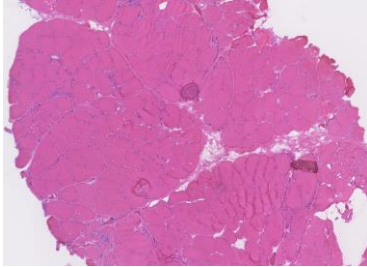
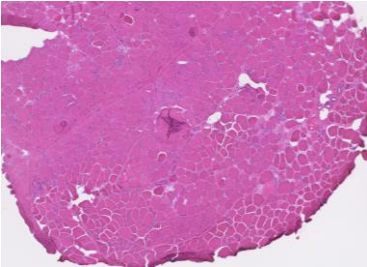
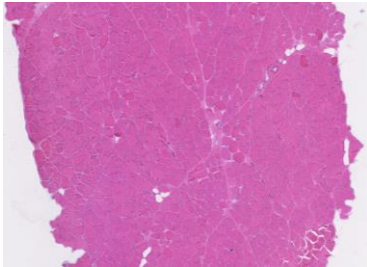
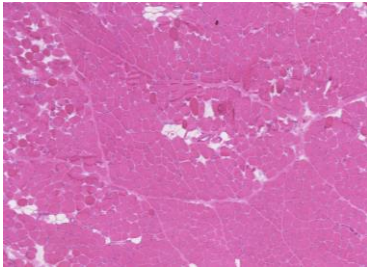
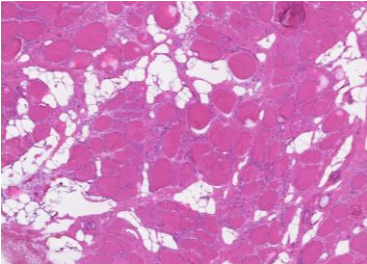
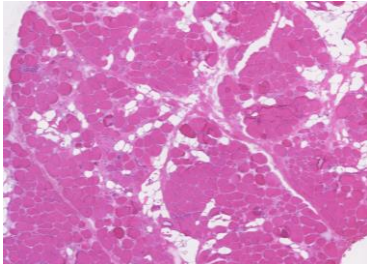
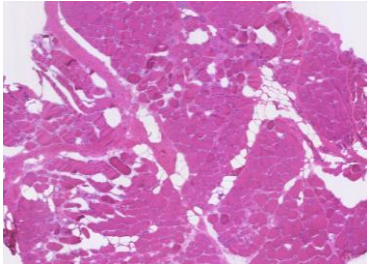
Pt 5: 18 months
(Age at baseline:
6.8 years)



Pt 6: 12 months
(Age at baseline:
7.7 years)



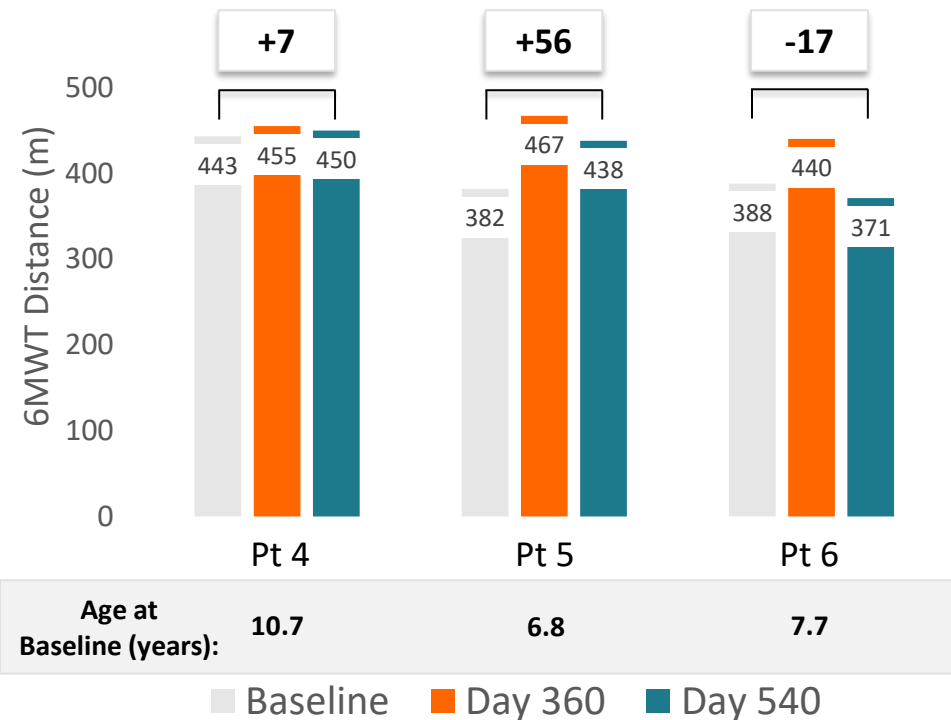
Limited Dystrophic Pathology Progression Observed in Long-Term Biopsies

	Baseline	Day 90	Last Timepoint	
Pt 4 (24 months)				Age at Last Timepoint: 12.7 yrs <i>Very mild active dystrophic pathology</i>
Pt 5 (18 months)				Age at Last Timepoint: 8.3 yrs <i>No active dystrophic pathology</i>
Pt 6 (12 months)				Age at Last Timepoint: 8.7 yrs <i>Very mild active dystrophic pathology</i>

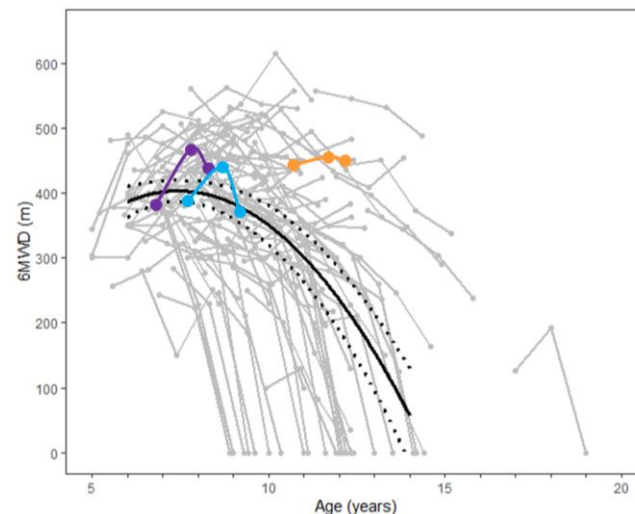
FUNCTIONAL ASSESSMENTS

6MWT Distances are Maintained 1.5 Years Post-Treatment

Mean Change from Baseline to Day 540: $+15.3 \pm 37.2$ m | Difference of +78.8 m Compared to Natural History over 1.5 Years



Individual Patient Trajectories



Data overlayed on Mercuri et al 2016

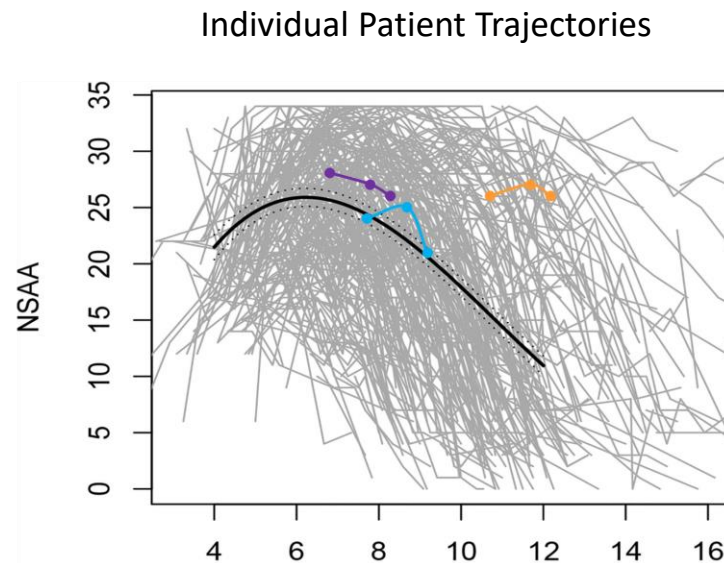
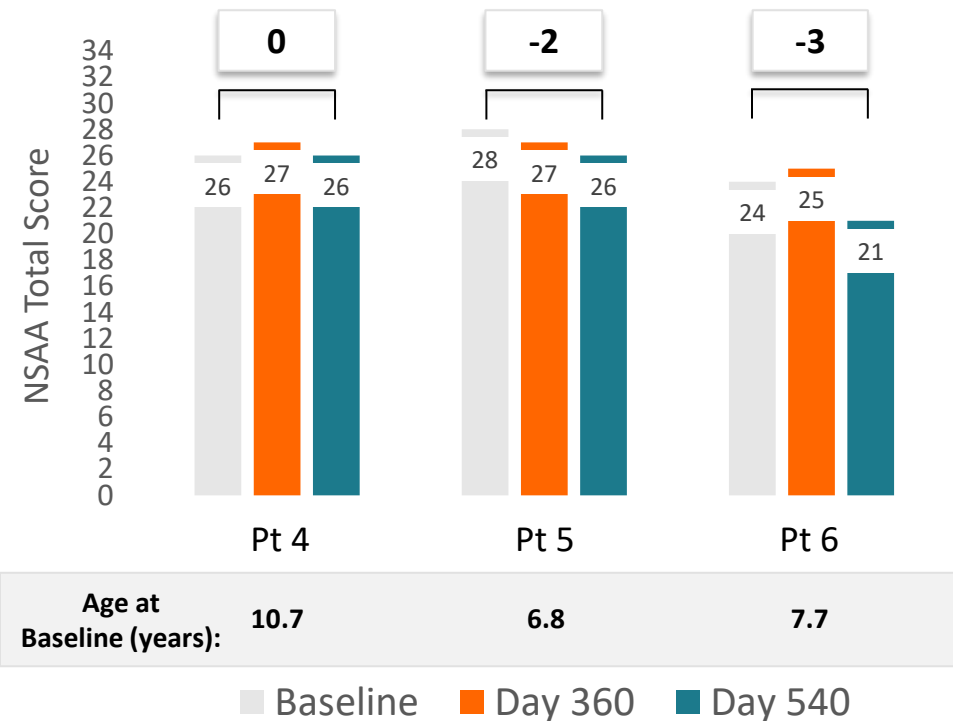
DMD Natural History

-63.5 m expected decline in 1.5 years after age 7

FUNCTIONAL ASSESSMENTS

NSAA Scores Show Minimal Change 1.5 Years Post-Treatment

Mean Change from Baseline to Day 540: -1.7 ± 1.5 Units | Difference of +2.8 Units Compared to Natural History over 1.5 Years

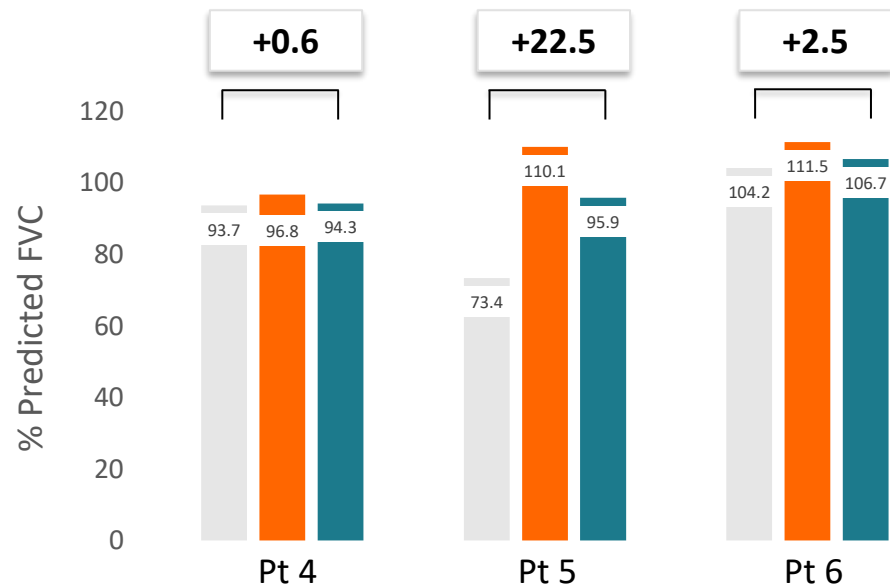


Data overlayed on Muntoni et al 2019
DMD Natural History
-4.5 unit expected decline over 1.5 years after age 6.3

FUNCTIONAL ASSESSMENTS

% Predicted FVC Continues to Show Stability or Improvement 1.5 Years Post-Treatment

Mean Change from Baseline to Day 540: $+8.5 \pm 12.1\%$ | Difference of +16.0% Compared to Natural History over 1.5 Years



Age at
Baseline (years):

10.7

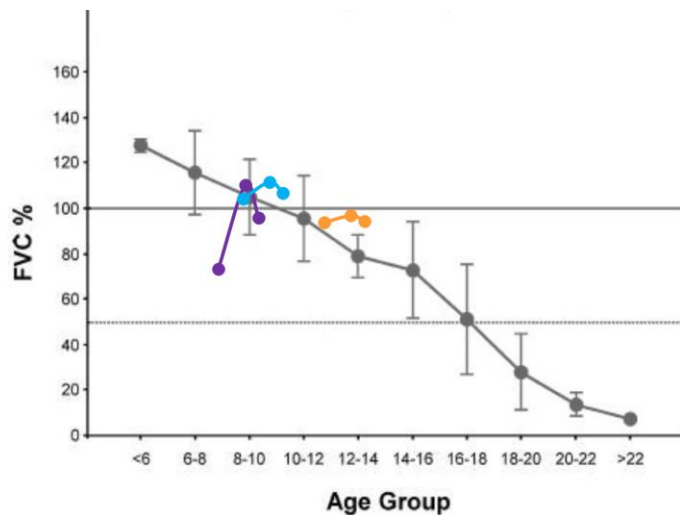
6.8

7.7

■ Baseline ■ Day 360 ■ Day 540

Interim data presented | FVC: forced vital capacity

Individual Patient Trajectories



Data overlayed on Mayer et al 2015

DMD Natural History

-7.5% expected decline over 1.5 years after age 6

FUNCTIONAL ASSESSMENTS

Stabilization Shown in PEF % Predicted and FEV1 % Predicted 1 Year Post-Treatment

Cohort	Subject	PEF % Predicted Baseline to 1 Year Change
Control	Ct 1	-1.1%
	Ct 2	n/a
	Ct 3	-18.2%
High Dose (2E14 vg/kg)	Pt 4	+15.9%
	Pt 5	n/a
	Pt 6	+26.7%

Cohort	Subject	FEV1 % Predicted Baseline to 1 Year Change
Control	Ct 1	-8.7%
	Ct 2	-17.0%
	Ct 3	-12.0%
High Dose (2E14 vg/kg)	Pt 4	+10.8%
	Pt 5	+15.5%
	Pt 6	+2.8%

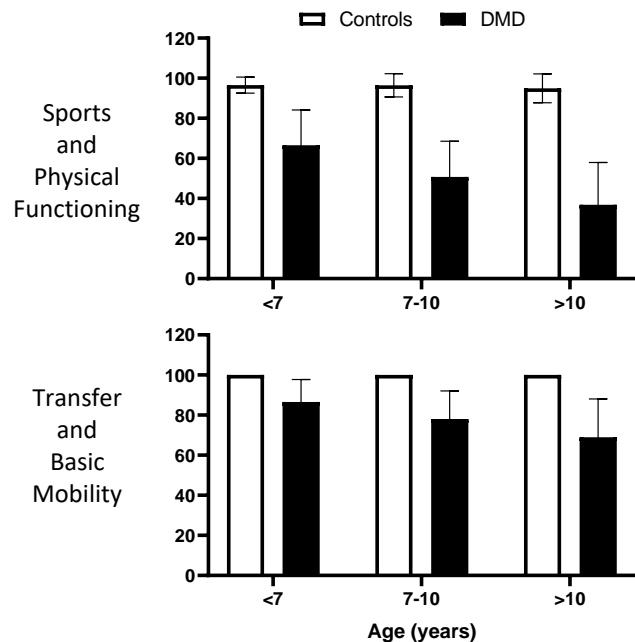
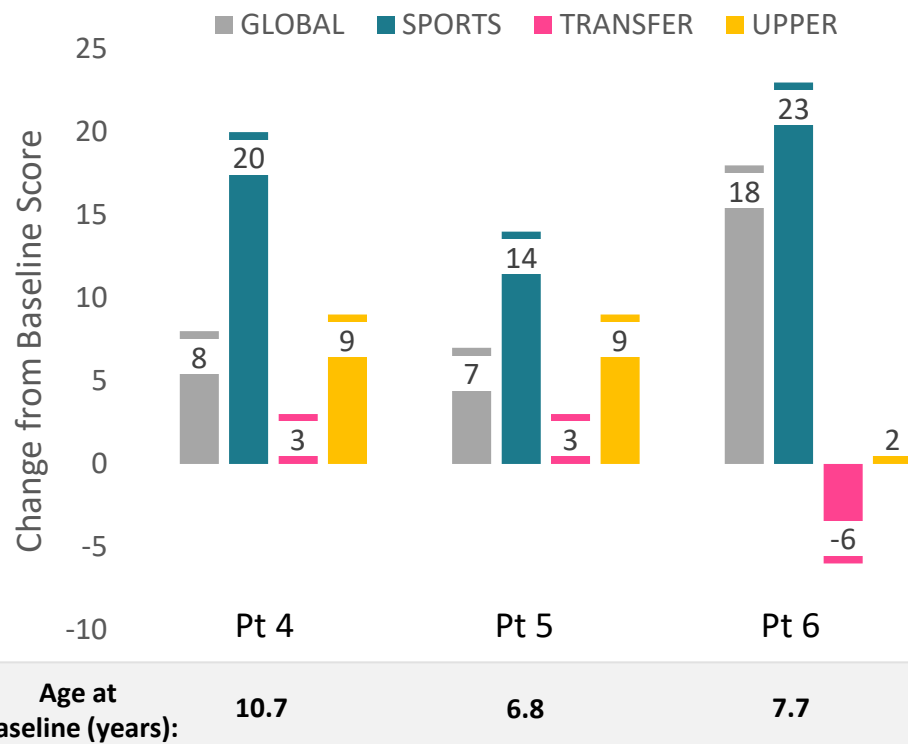
Natural history analyses of pulmonary function data suggest that patients would normally expect to exhibit a decline over the same time periods assessed

PEF: peak expiratory flow | FEV1: forced expiratory volume in one second

n/a values represent either baseline or endpoint assessments not available or identification of suboptimal maximal respiratory efforts during testing

PATIENT REPORTED OUTCOMES

Sustained Meaningful Improvements in SGT-001 Treated Subjects at 1.5 Years by PODCI



Modified from McDonald et al 2010, Henricson et al 2013
DMD Natural History

- 7.6 point expected decline over 1.5 years in Global scale
- 4.7 point expected decline over 1.5 years in Sports scale
- 14.9 point expected decline over 1.5 years in Transfer scale

Key Takeaways From Interim Analysis of IGNITE DMD



Durable expression and function of microdystrophin protein in biopsies collected ≥ 12 months post-administration of SGT-001

- ✓ Sustained or increased microdystrophin protein levels and percent positive muscle fibers
- ✓ Sarcolemmal restoration of key dystrophin associated proteins β -sarcoglycan and nNOS



Encouraging evidence of functional benefit shown post-treatment vs natural history

- ✓ 6MWT Distances are Maintained 1.5 Years Post-Treatment
- ✓ NSAA Scores Show Minimal Change 1.5 Years Post-Treatment
- ✓ % Predicted FVC Continues to Show Stability or Improvement 1.5 Years Post-Treatment
- ✓ Stabilization Shown in PEF % Predicted and FEV1 % Predicted 1 Year Post-Treatment



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

- ✓ Sustained Meaningful Improvements in SGT-001 Treated Subjects at 1.5 Years by PODCI

Totality of data supports continued dosing in IGNITE DMD at 2E14 vg/kg dose

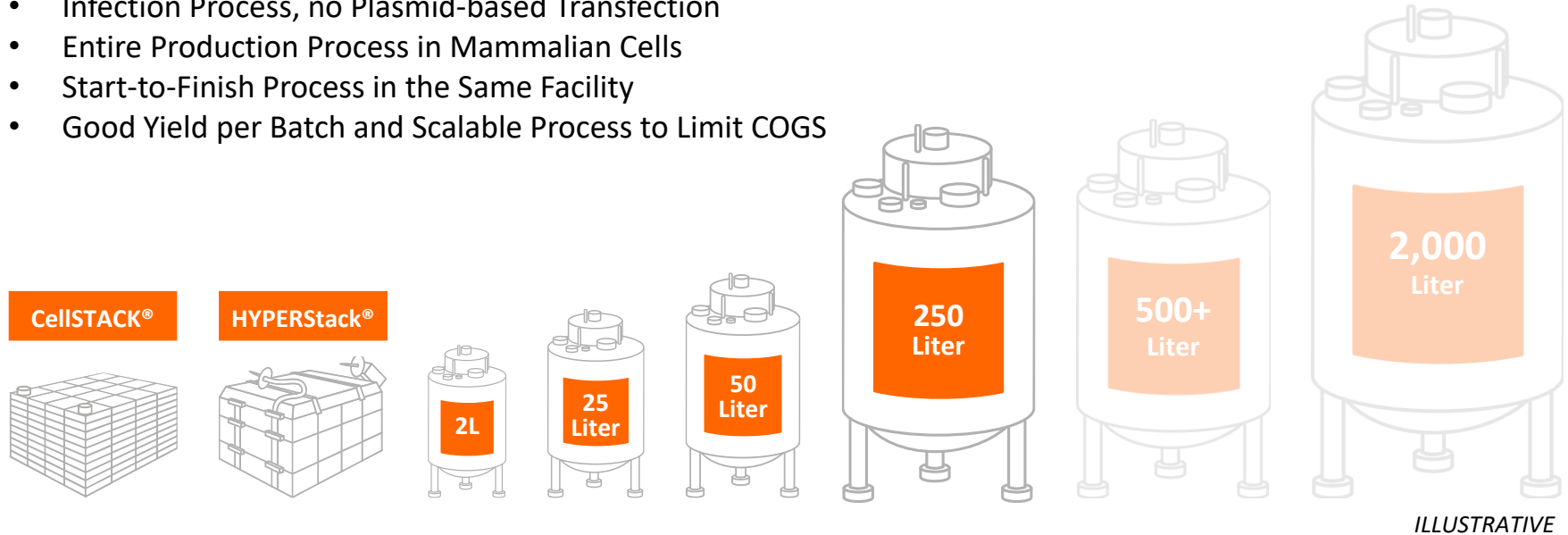


SOLID
BIOSCIENCES

SGT-001 Manufacturing

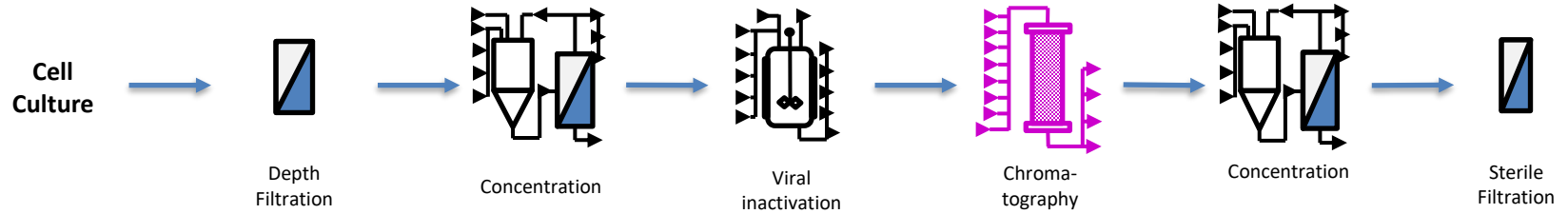
HSV-Based Process is an Integrated and Scalable Approach to AAV Manufacturing

- Infection Process, no Plasmid-based Transfection
- Entire Production Process in Mammalian Cells
- Start-to-Finish Process in the Same Facility
- Good Yield per Batch and Scalable Process to Limit COGS



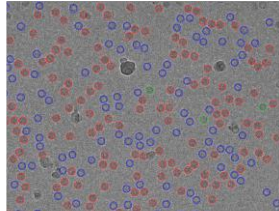
- Solid has successfully scaled up production to 250L in suspension and produced multiple GMP batches
- Production utilizes proven, validated and widely-available standard bioreactors

2nd Generation Manufacturing Process (2020): Reduced Empty Capsids and Total Viral Load



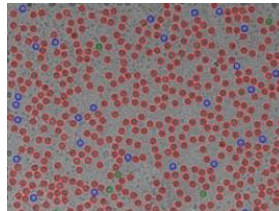
% of Full vs Empty Capsids

50% full



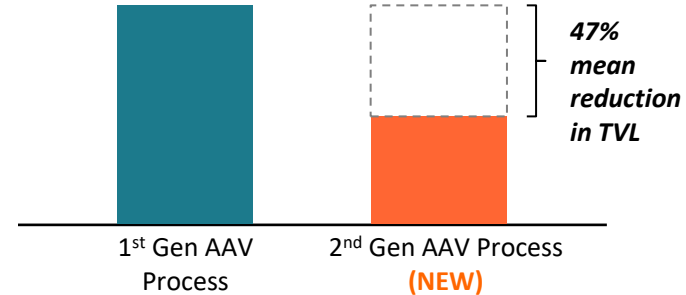
1st Gen AAV Process

>90% full



2nd Gen AAV Process
(NEW)

Total Viral Load at 2e14 vg/kg

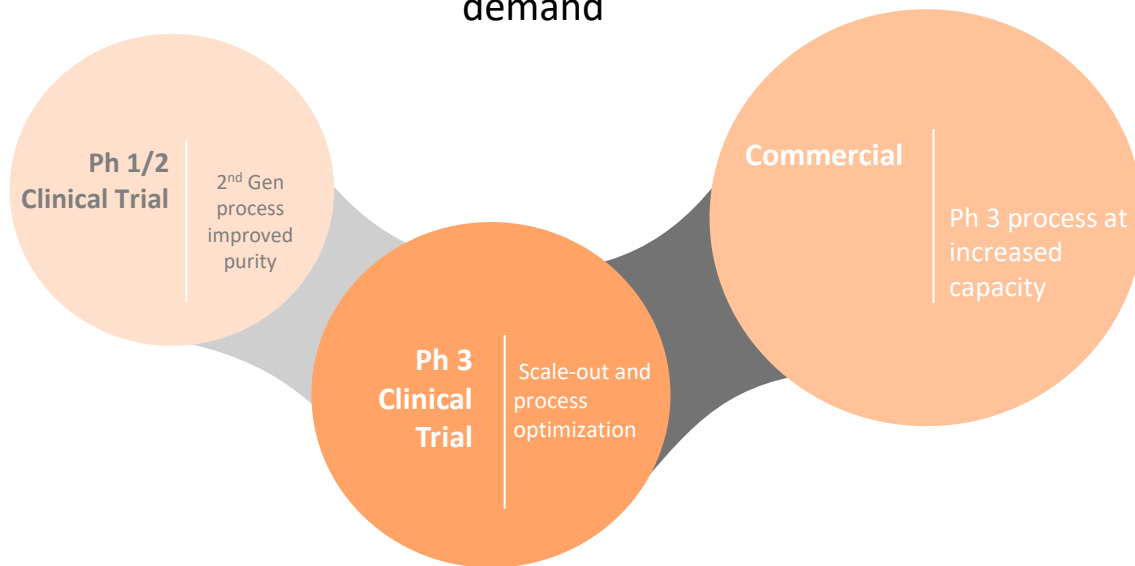


Improvement from 50% to 90% full capsids in final drug product

Manufacturing Strategy to Support Pivotal Study and Commercial Launch

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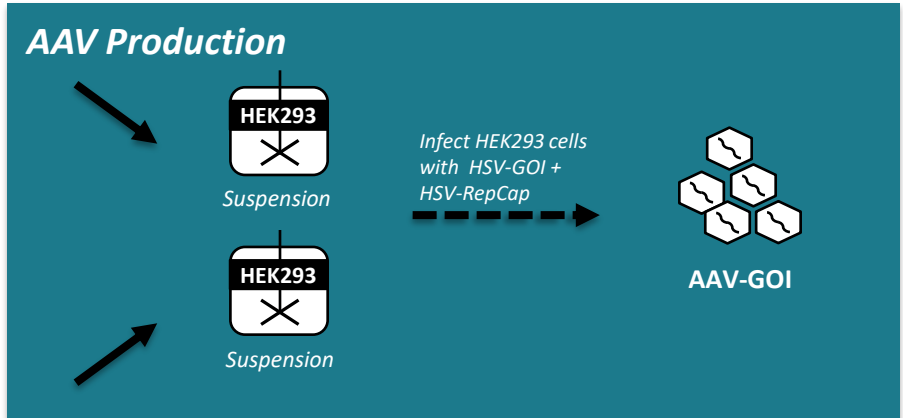
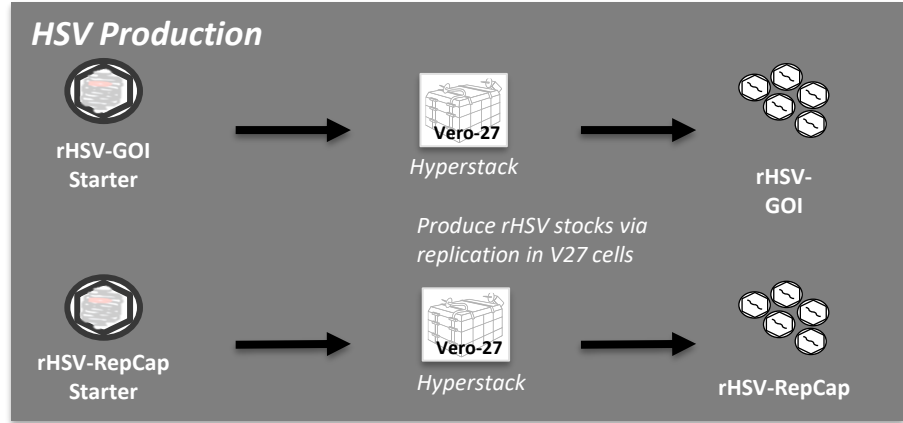
Solid's current manufacturing process produces high quality product and is scalable to meet additional demand



Solid's current manufacturing process is evolving in preparation for Phase 3 and Commercial production based on extensive understanding of the second-generation process and yield optimization strategy

HSV Manufacturing: Maintain Current Process while Scaling to Meet Phase 3 and Commercial Needs

- Optimizes manufacturing floor time and downstream purification yields
- Maintains low number of bioreactors to support operational efficiencies
- Focus on change in process steps that gain high return with low compliance risk



Pipeline Update

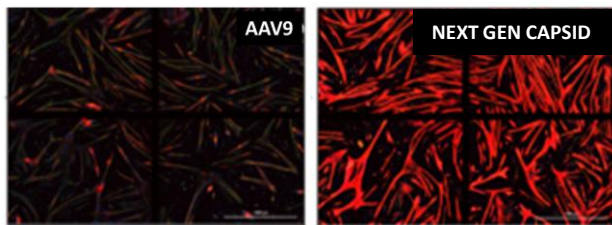
SGT-003

Ultragenyx Collaboration

Development of Next Generation Capsids Designed to Enhance Muscle Transduction Efficiency

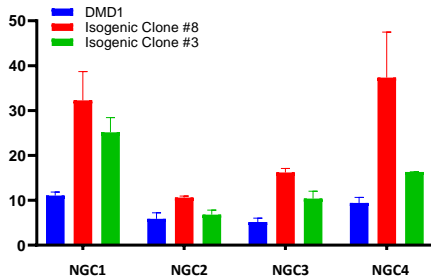
Capsid Library Development and In Vitro Screening

C2C12 Microdystrophin Protein Expression



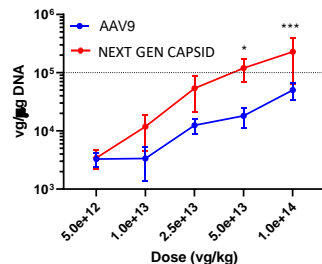
Human DMD Cell Microdystrophin Expression

Fold Change over AAV9

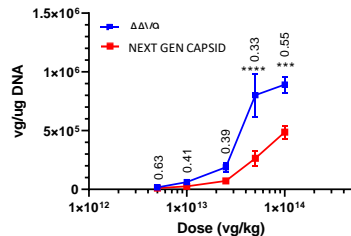


Biodistribution (vg/ μ g gDNA) in Skeletal Muscle and Liver

3-7-fold increase in biodistribution in skeletal and cardiac muscle

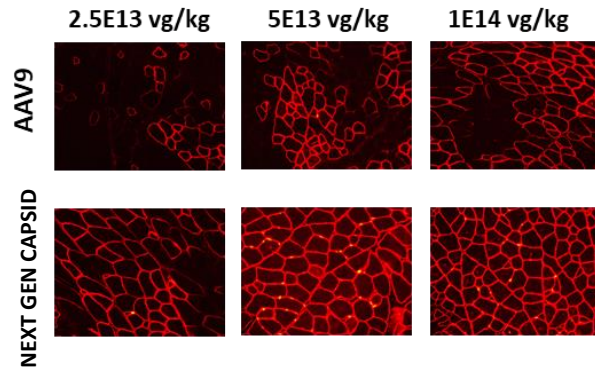


2-3-fold decrease in biodistribution to liver

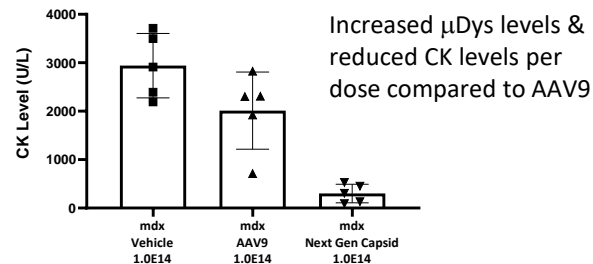


Disease Model (mdx) Testing

IF Imaging of mdx Quads



Serum CK Day 29 Post-Treatment



Increased μ Dys levels & reduced CK levels per dose compared to AAV9

Strategic Partnership with Forge Biologics to Develop and Manufacture SGT-003

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- SGT-003: preclinical candidate that combines a next-generation and rationally designed capsid candidate with Solid's proprietary nNOS-containing microdystrophin
- Forge will provide an AAV vector process, scale-up engineering and cGMP manufacturing services for SGT-003. The program will employ Forge's Blaze Vector™ production platform and proprietary HEK293 suspension Ignition Cells™ and pEMBR™ adenovirus helper plasmid
- Forge's integrated platforms and cGMP gene therapy manufacturing capabilities coupled with Solid's in-depth knowledge in high dose gene therapy development and manufacturing will introduce an additional method to produce AAV gene therapy at Solid, and help to accelerate human proof of concept for SGT-003

Collaboration to Advance Next Generation DMD Constructs



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



- 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

Key Takeaways

Solid has amassed assets, experience and data from our lead DMD gene therapy program, SGT-001

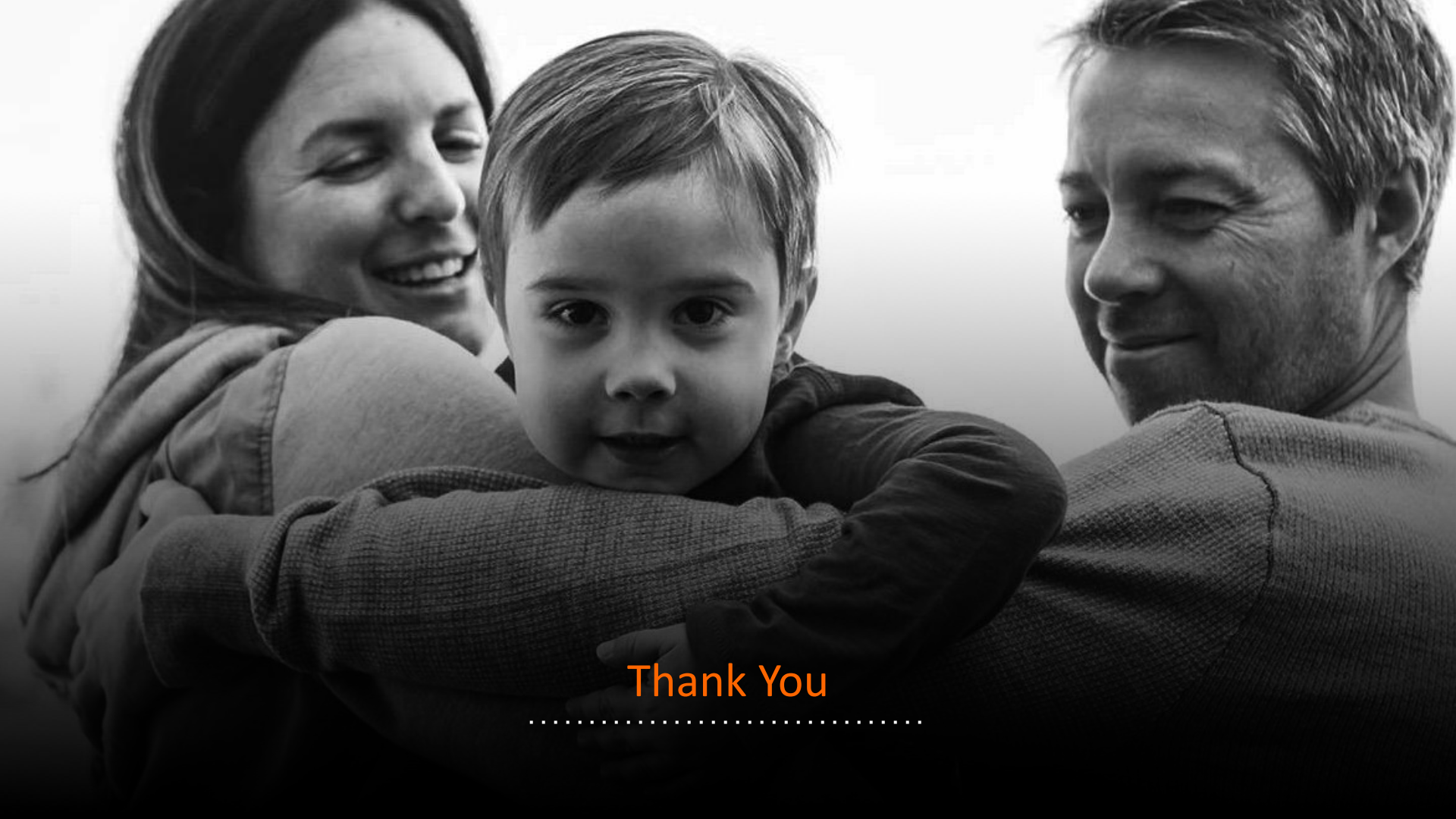
- Created a unique and distinctive transgene construct
- Generated robust preclinical protein expression and activity across multiple species
- Identified an effective dose with signals of activity from early clinical results
- Began scalable HSV suspension manufacturing process that has the potential to drive attractive gross margins

Solid has optimized our manufacturing process delivering SGT-001 dose with 50% reduction in total viral load, designed to enhance safe and effective dosing

Solid is developing a pipeline offering multiple opportunities to deliver our differentiated microdystrophin construct to patients

Solid is positioned for success

- Financial runway will enable Solid to advance key strategic priorities
- Partnerships that complement and strengthen our areas of expertise



Thank You

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