



# **Q1 2022 Business Update and Financial Results**

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April 27, 2022

# Financial Information and Forward-Looking Statements

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This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the reduction in headcount, reduction in corporate expenses 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.

# Introductions

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**Ilan Ganot**

*Co-Founder, President  
and Chief Executive Officer*



**Joel Schneider, PhD**

*Chief Operating Officer*



**Carl Morris, PhD**

*Chief Scientific Officer*



**Roxana Donisa Dreglici, MD**

*Senior Vice President,  
Head of Clinical Development*



**Caitlin Lowie**

*Vice President, Communications  
and Investor Relations*

# AGENDA

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- Strategic Update to Develop SGT-001 and SGT-003
- SGT-001 Manufacturing and IGNITE DMD Update
- SGT-003 and Novel Capsid Preclinical Data
- Q&A

# STRATEGIC UPDATE:

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Aligning our resources behind Solid's core values of innovation and patient centricity, with a focus on bringing our differentiated microdystrophin to more patients

# Updated 2022 Strategic Priorities

## Strategic refocus designed to rapidly bring SGT-001 and SGT-003 to patients

### Advance SGT-001 with new manufacturing process

- IGNITE DMD functional and durability data supports program advancement
- Program is transitioning to a commercially scaled transient transfection-based manufacturing process with clinical entry in 1H 2023

### Accelerate SGT-003 pipeline program to the clinic

- Early 2023 IND submission anticipated
- Novel capsid development continues following release of new preclinical data in NHP exploratory study
  - $\geq 2X$  increases in muscle targeting, decreased liver uptake
  - $\geq 10X$  increase in reporter gene expression in muscle and heart

### Solid is positioned for success

- Strategic program and manufacturing process alignment supports funding of operations through important clinical milestones and into Q2 2024
- Organizational realignment results in  $\sim 35\%$  headcount reduction
- \$180m in cash and investments as of Mar 31, 2022





**SGT-001 /  
IGNITE DMD**

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# Key Takeaways From Interim Analysis of IGNITE DMD

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## 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 all patients in 2E14 vg/kg cohort show consistent levels of expression
- ✓ 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**



# Solid to Transition SGT-001 Manufacturing to Commercially Scaled Process



*SGT-001 to continue via transient transfection-based manufacturing, with minimal impact to potential BLA timeline*

**Highly potent drug product demonstrating high levels of microdystrophin expression in *in vitro* and *in vivo* testing**

**High quality product**

**Consistent product supply**

**Access to a broader supply chain**

Using a transient-based process may provide improvements to manufacturability as well as additional organizational efficiencies by streamlining to a single manufacturing methodology

# Solid's Path Forward for SGT-001

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## Manufacturing

Solid to transition to new process with target to have product available in early-2023

## IGNITE DMD

Solid has decided to conclude enrollment in IGNITE DMD and transition to FDA engagement and planning for future clinical activities. Solid to continue monitoring patients for 5-years post-treatment and release functional data

## Clinical Development

Solid to refine clinical development strategy with future patients being dosed with SGT-001 produced via transient production. Solid to initiate Natural History Study

Solid expects to initiate dosing patients in 2023, following GMP production and FDA discussions

# SGT-003 AND NOVEL CAPSIDS

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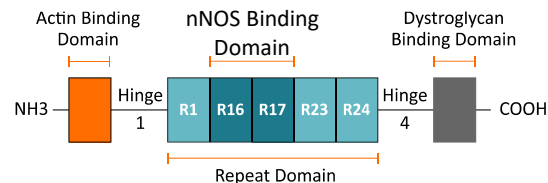
# SGT-003 is Rapidly Moving to the Clinic, Leveraging Learnings from SGT-001 to Reduce Immunological Burden and Increase Muscle Delivery

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## 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

Microdystrophin function and expression data from the IGNITE DMD clinical study supports continued SGT-003 development

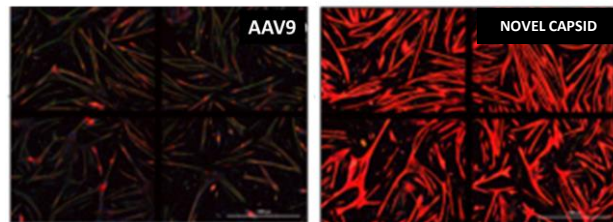
# Compelling Data Resulted in Advancement of SGT-003 Program

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

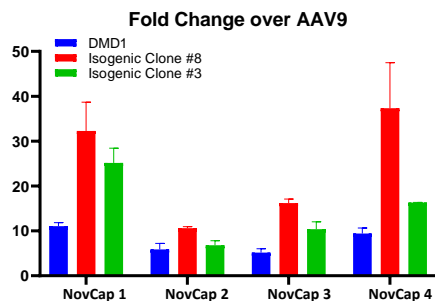
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## Cell Based *In Vitro* Assays WT Mouse and Human Dystrophic Cells

### C2C12 Microdystrophin Protein Expression

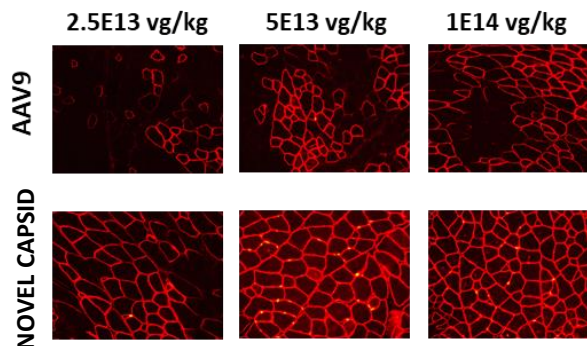


### Human DMD Cell Microdystrophin Expression

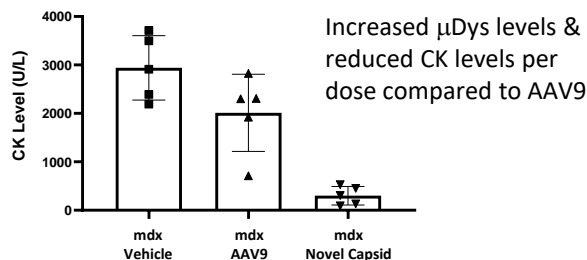


## *In Vivo* Disease Model (mdx) Testing

### IF Imaging of mdx Quads

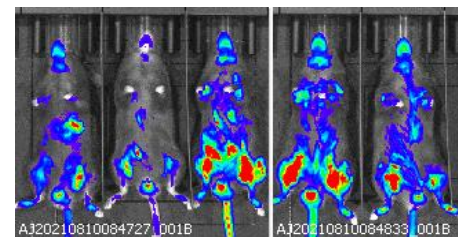


### Serum CK Day 29 Post-Treatment

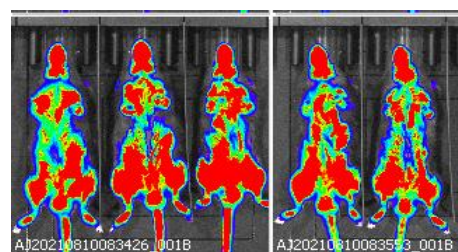


## *In Vivo* WT Mouse Reporter

### AAV9 3E14 vg/kg



### Novel Capsid 3E14 vg/kg



Advanced to Program status: SGT-003

# Confirming Earlier Preclinical Data in a Non-Human Primate (NHP) Exploratory Study

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## Program Goal

Improve expression at a lower dose, allowing for decreased total viral load to patients

## Microdystrophin Mouse Studies

Novel capsids show increases in muscle biodistribution and microdystrophin expression compared to AAV9 in dystrophic animals

## Novel Capsid Mouse Models

Novel capsid library demonstrates improved muscle tropism in multiple animal models



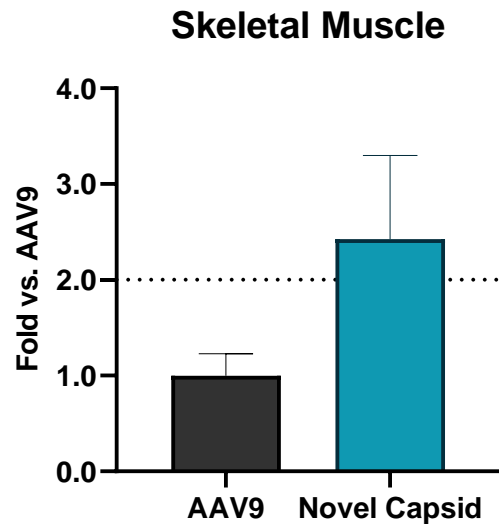
## NEW DATA - NHP Exploratory Study

Confirm safety and expression; Eight 2-year-old NHPs, 4 male / 4 female; 4 dosed with AAV9 / 4 dosed with Solid's novel Capsid

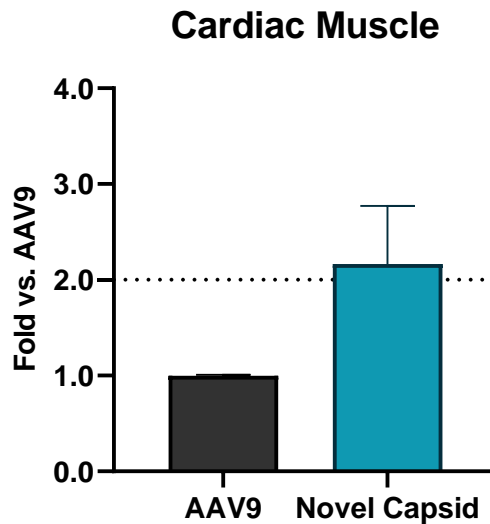


# NHPs Administered Novel Capsid Show Increased Muscle Biodistribution and Decreased Biodistribution to Liver Relative to AAV9

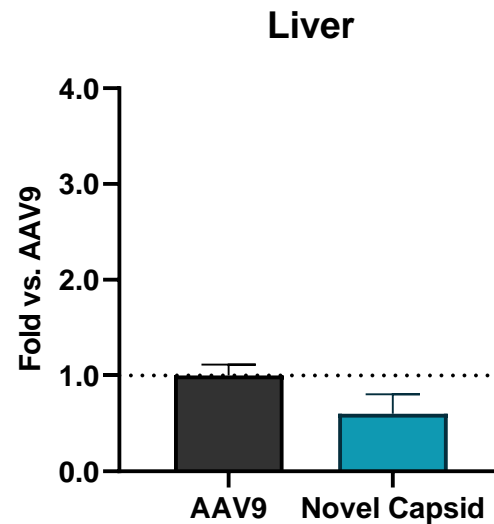
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>2X vs AAV9



>2X vs AAV9

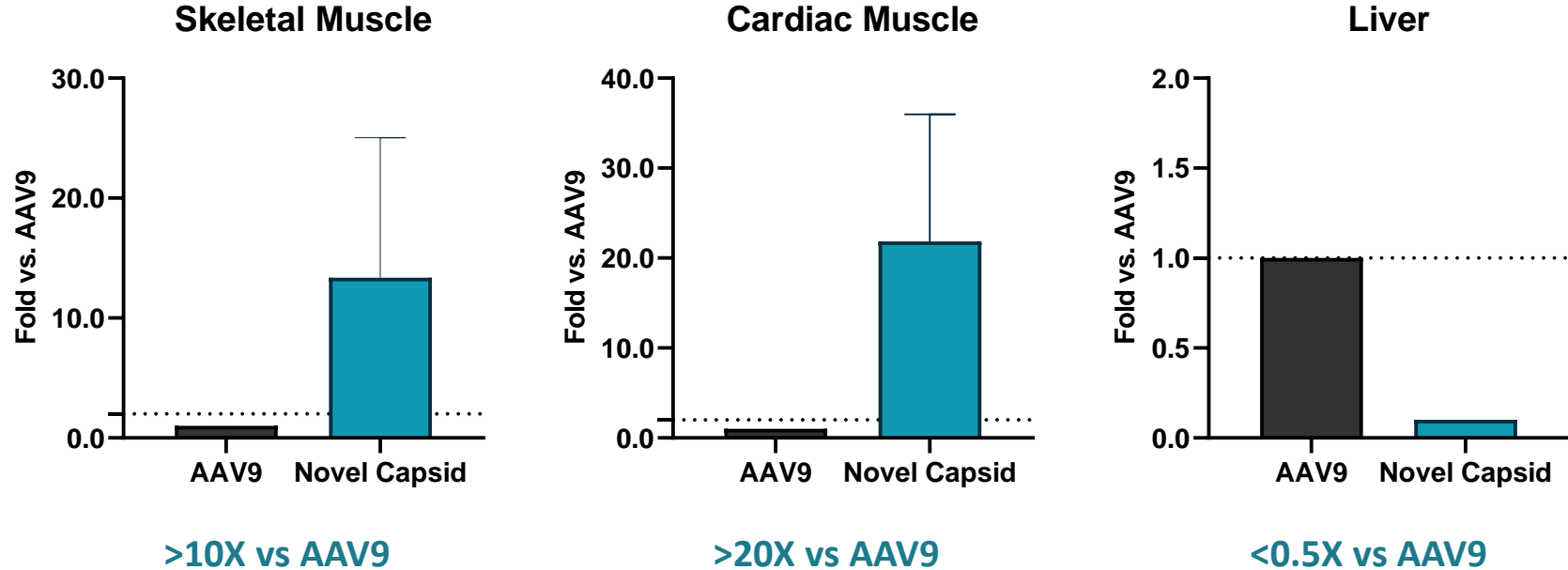


>0.5X vs AAV9

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

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

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

# SGT-003 Moving Towards and Early 2023 IND Submission

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## **SGT-003: Combining a Novel Capsid with Solid's Differentiated Microdystrophin**

- Advancing rapidly, working with CDMO to utilize the novel capsid within the SGT-003 program to get a next-generation microdystrophin construct in the clinic next year
- Translatability activities across species continue, along with other pre-IND enabling research activities
- Evaluation of exploratory studies and other research activities are ongoing that could be applicable to other muscle-related disorders

Novel, next generation capsid combined with Solid's microdystrophin, which has been confirmed clinically in IGNITE DMD, is moving quickly to clinical proof of concept in SGT-003

A close-up photograph of a woman with long dark hair holding a baby in a black car seat. The baby has light brown hair and blue eyes, looking directly at the camera. The woman is wearing a red jacket with a black logo on the sleeve. The background is a blurred outdoor setting with green foliage.

**DRIVING  
THE FUTURE**

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# Positioned for Success

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## Strategy

Refinement of corporate strategy to focus organization around advancing differentiated microdystrophin through two promising programs

## Organization

Consolidation of resources leveraging single manufacturing platform and focused strategy resulted in a headcount reduction of approximately 35%

## Financial

\$180m cash and cash equivalents enable Solid to fund its operating expenses through important clinical milestones and into the second quarter of 2024



**THANK YOU**

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