Forward-Looking Statements

This presentation includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, which involve a number of risks and uncertainties. These forward-looking statements include all matters that are not historical facts and, without limiting the foregoing, can be identified by the use of forward-looking terminology, including the terms “believe,” “estimate,” “project,” “anticipate,” “expect,” “seek,” “predict,” “continue,” “possible,” “intend,” “may,” “might,” “will,” “could,” “would” or “should” or, in each case, their negative, or other variations or comparable terminology. They appear in a number of places throughout this presentation and include statements regarding our intentions, beliefs or current expectations concerning, among other things, our product candidates, research and development and clinical trial plans, manufacturing plans, commercialization objectives, prospects, strategies, the industry in which we operate and potential collaborations. We derive many of our forward-looking statements from our operating budgets and forecasts, which are based upon many detailed assumptions. While we believe that our assumptions are reasonable, we caution that it is very difficult to predict the impact of known factors, and, of course, it is impossible for us to anticipate all factors that could affect our actual results. For a discussion of potential risks and uncertainties, and other important factors, any of which could cause our 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 our most recent filings with the Securities and Exchange Commission. All forward-looking statements included in this presentation represent our views as of the date hereof and should not be relied upon as representing our views as of any date subsequent to the date on the cover page of this presentation. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we specifically disclaim 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.
Presentation Overview

• Introduction to Solid Biosciences
• Background on dystrophin
• Gene transfer for the treatment of Duchenne
• SGT-001 manufacturing
• Solid’s SGT-001 microdystrophin gene therapy program and the IGNITE DMD clinical trial
Purpose-Built to Solve Duchenne Muscular Dystrophy (DMD)

**360-Degree Approach**
Address all facets of DMD

**Differentiated Lead Gene Transfer Program**
Data from second dose cohort later this year

**Scalable Manufacturing Process**
Meet clinical and commercial needs
Solid Is Addressing the Full Spectrum of Duchenne

**CORRECTIVE THERAPIES**
- Gene therapy to address the genetic cause of DMD

**DISEASE-MODIFYING THERAPIES**
- Small molecules and biologics to address disease mechanisms

**DISEASE UNDERSTANDING**
- Biomarkers and endpoints to improve development

**ASSISTIVE DEVICES**
- Technology to support mobility
Corrective Therapies

Microdystrophin Gene Transfer
Dystrophin Function in Healthy Muscle

- Dystrophin protects the muscle from damage and stabilizes critical dystrophin-associated proteins.
Dystrophin is Missing in DMD Muscle

- In DMD, mutations in the dystrophin gene result in the absence of functional dystrophin protein.
- Muscle fibers become damaged, cannot be repaired or replaced and are taken over by fat and scar tissue.
Gene Transfer to Address the Genetic Cause of DMD

- Gene transfer brings instructions to the cell to make a new kind of dystrophin designed to replace the missing dystrophin protein.
What Is Gene Transfer For DMD?

Gene transfer for DMD is made up of three essential elements:

1. Gene
2. Promoter
3. Vector

The combined product is then given to the patient.

1. **Gene**
2. **Promoter**
3. **Vector**

*Carries the gene*

*Controls expression*
Each Component of SGT-001 Was Carefully Selected

- **Transgene**: Restore key functions of a complex protein
  - **SGT-001 microdystrophin gene**

- **Promoter**: Expression in skeletal and heart muscle
  - **CK8 promoter**

- **Vector**: Targets skeletal and heart muscle
  - **AAV9 vector**
SGT-001 AAV-Mediated Microdystrophin Gene Therapy
Features of SGT-001 Microdystrophin

Full Length Dystrophin Protein

SGT-001 Microdystrophin Protein

- SGT-001 selection based on more than 30 years of research; confirmed through comparison experiments by Solid
Animal Studies Show SGT-001 Microdystrophin is Made Selectively in Muscle

<table>
<thead>
<tr>
<th>Dose (vg/kg)</th>
<th>0</th>
<th>1E14</th>
<th>2E14</th>
<th>1E13</th>
</tr>
</thead>
</table>

**SGT-001 Treated Diaphragm**

<table>
<thead>
<tr>
<th>Tissue</th>
<th>Dystrophin</th>
<th>Microdystrophin</th>
</tr>
</thead>
<tbody>
<tr>
<td>Diaphragm</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Liver</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Vastus Lateralis</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Company data. Three month efficacy study in GRMD canines. Representative only.
SGT-001 Microdystrophphin with nNOS Binding Domain Showed Greater Improvements in Muscle Strength in a Mouse Model of DMD

SGT-001 treatment led to force generation levels comparable to those in wild-type mice

Specific diaphragm force 6 months post-treatment. Data shown as mean ± SEM. n=5-7 per group.
Microdystrophin Expression Lasts at Least 2.5 years in an Animal Model of DMD

Manufacturing

Producing SGT-001
Addressing the DMD Gene Therapy Supply Challenge

**HIGH PATIENT NEED** × **HIGH AVERAGE PATIENT WEIGHT** × **HIGH DOSES** = **SIGNIFICANT SUPPLY NEEDS**

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**Solid Manufacturing Capability**

- Successfully scaled up to 250L in suspension and produced multiple batches
- Each 250L batch can dose multiple patients
- Create ability to potentially treat 1,000s of patients
GMP Manufacturing Process Currently Producing At Significant Volume

- Successfully scaled up to 250L in suspension and produced multiple batches
- Each 250L batch can dose multiple patients
- Utilizes proven, validated and widely-available standard bioreactors

Successful scale up to 250L suspension complete
Scaling Process To Efficiently Supply Commercial Markets

- Continue to optimize process development in Solid labs
- Maintain low number of bioreactors to support operational efficiencies
- Create ability to potentially treat 1,000s of patients

Successful scale up to 250L complete

Further scale if needed
Original study was a randomized, controlled, open-label, single-ascending dose study.

**Primary Endpoints:**
- Safety
- SGT-001 microdystrophin expression at 12 months

**Secondary Endpoints:**
- Muscle function and strength
- Cardiac and respiratory function
- Muscle mass area and composition (MRI)

**Key Details:**
- **Dose Escalation:**
  - Started at $5 \times 10^{13}$ vg/kg, $n=3$
  - Dose escalated to $2 \times 10^{14}$ vg/kg, ongoing

- **Matched Control Group**

- **Observation**

- **Delayed Treatment**: To be initiated
IGNITE DMD: Study Status/Updates

Announced preliminary three-month muscle biopsy data for first three patients at the starting dose of SGT-001 (5E13 vg/kg)

- Low levels of microdystrophin protein expressed via immunofluorescence in all three patients
- In one patient, microdystrophin was detected via western blot (<5%) and ~10% of muscle fibers via immunofluorescence

Necessary steps were completed to dose escalate SGT-001 to 2E14 vg/kg in a second cohort of patients

Announced dosing of first patient in second cohort (2E14 vg/kg) and initiation of clinical trial activities at two additional sites

- Transient decline in platelet count, which fully resolved
- Transient abnormalities on liver function tests, which quickly responded to an increased dose of oral steroids
- A gastrointestinal infection unrelated to study drug

Announced protocol amendment and dosing of second patient in second cohort (2E14 vg/kg)

- Established an upper weight limit of 25kg for at least the next subject dosed at 2E14 vg/kg
- No control arm for the rest of the IGNITE DMD second cohort at 2E14 vg/kg

Data from second cohort expected later this year
Thank you!

@SolidBioDMD
#TogetherWeAreSolid
#PatientPowered