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, 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.
Solid Biosciences: Committed To Curing DMD

Singular Focus on DMD
• 360-degree approach to targeting all aspects of DMD
• Focused on every patient, regardless of DMD mutation, age or stage of disease progression

Center of Excellence
• Experienced management team
• World-renowned advisors

Founded by a Family
• Ilan and Annie Ganot

Significant Partnerships with the Advocacy Community
• DMD patients heart and center of our company
• Vast network of advocacy relationships around the globe

Resourced for Growth
• Public company in Jan 2018
• New headquarters and labs
• Team of 80 employees and growing
What is gene transfer for DMD?

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

1. **Gene**
2. **Vector** *Carries the gene*
3. **Promoter** *Controls expression*

The combined product is then administered to the patient.
SGT-001 is Specially Designed

- Based on more than 30 years of work by top scientists (Drs. Jeffery Chamberlain and Dongsheng Duan)
- Built with the latest advances in microdystrophin biology
- Carefully studied in a robust preclinical program for DMD
IGNITE DMD: Phase I/II Adaptive Clinical Trial

Safety (primary endpoint)

Efficacy (primary endpoint)
• Microdystrophin expression

Efficacy (secondary endpoints)
• Muscle function and strength
• Cardiac and respiratory function
• Muscle mass area and composition (MRI)

Randomized, controlled, open-label, single-ascending dose

Plan to enroll 16-32 non-ambulatory adolescents and ambulatory children

Clinical trial initiated at the University of Florida
IGNITE DMD Clinical Trial Design

**Cohort 1**
Adolescents & Children

**Cohort 2**
Adolescents &/or Children

**Delayed Treatment Group**

**Switch to Treatment**

**Screening, Baseline & Randomization**
1:1 (n=16 to 32)

**Starting dose:** $5 \times 10^{13}$ vg/kg

**Potential to dose escalate**

**Three Biopsies:**
- At baseline (before SGT-001 administration)
- At 12 months
- And at an intermediate timepoint (45 days, or 3, or 6 or 9 months)
Inclusion and Exclusion Criteria

**Inclusion Criteria:**

- Clinical diagnosis of DMD and documented dystrophin gene mutation predictive of DMD
- Anti-AAV9 antibodies below pre-specified thresholds
- Must have stable cardiac and pulmonary function
- Ambulatory children and non-ambulatory adolescents by pre-specified criteria
- Stable daily dose of oral corticosteroids ≥ 24 weeks

**Exclusion Criteria:**

- Any pre-existing conditions or abnormalities that would impact patient safety, compromise completion of treatment and follow-up, or impair assessment of study results
- Exposure to another investigational drug within 3 months prior to screening (or longer, depending on the investigational drug)
- Exposure to drugs affecting dystrophin or utrophin expression within 6 months prior to screening
Status

• Dosed the first adolescent patient in February 2018

• FDA placed IGNITE DMD on clinical hold in March due to a Serious Adverse Reaction

• The event fully resolved and the patient returned to his normal activities

• Dr. Barry Byrne & his team at the University of Florida provided exceptional patient care

• FDA lifted the clinical hold and we are reinitiating clinical activities

• We plan to dose several children prior to dosing more adolescents
Committed To Patients With Duchenne Muscular Dystrophy
Thank You