# Solid Biosciences Presents Data from Duchenne Muscular Dystrophy Gene Therapy Program at ASGCT 23rd Annual Meeting

May 14, 2020

*Virtual oral presentation reviews interim clinical biomarker data for SGT-001 and provides data on expression and co-localization of microdystrophin and dystrophin associated proteins in patients in the IGNITE DMD trial*

*Virtual poster presentation provides data characterizing novel adeno-associated virus (AAV) vectors engineered for muscle gene delivery*

CAMBRIDGE, Mass., May 14, 2020 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) today announced the presentation of clinical biomarker data from its SGT-001 microdystrophin gene therapy clinical trial for the treatment of Duchenne muscular dystrophy (Duchenne) on May 13 at the American Society of Gene and Cell Therapy (ASGCT) 23rd Annual Meeting, which is being held virtually. In addition, data from studies characterizing novel adeno-associated virus (AAV) vectors engineered for muscle gene delivery were presented in a poster session.

**Presentation Information**

*Abstract Number: 500*
SGT-001 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy. Oral presentation delivered by Patrick Gonzalez, PhD, Associate Director, R&D, Solid Biosciences.

Data presented from the ongoing IGNITE DMD clinical trial studying SGT-001 at a dose of 2E14 vg/kg were:

- SGT-001 administration results in dose-dependent, muscle wide microdystrophin expression in muscle biopsies collected 90 days post-SGT-001 administration.
- SGT-001-driven microdystrophin expression results in stabilization of dystrophin associated proteins.
- SGT-001-driven microdystrophin expression results in restored enzymatically active neuronal nitric oxide synthase (nNOS) at the sarcolemma.

*Abstract Number: 558*
Characterization of Novel AAV Vectors Engineered for Muscle Gene Delivery. Poster presentation and online Q&A delivered by Jennifer Green, PhD, Senior Scientist, R&D, Solid Biosciences.

This study utilized a rational design approach to generate a set of novel capsids predicted to have increased muscle tropism and transduction efficiency for the development of treatment for Duchenne. Key findings are:

- AAV-SLB101, a novel capsid, showed superior transduction efficiency in comparison to AAV9 in *in vitro* assays in both mouse and Duchenne human skeletal muscle cells.
- These *in vitro* results translated to increased biodistribution and microdystrophin protein expression *in vivo* in both quadriceps and heart, and decreased biodistribution to liver, in comparison to AAV9.
- An expanded panel of novel capsids identified two more candidates of interest, AAV-SLB102 and AAV-SLB111, that look similar to AAV-SLB101 in *in vitro* assays for binding, uptake and microdystrophin protein expression in C2C12 cells.

The presentation and poster are available on the Scientific Publications & Presentations page of the Solid Biosciences corporate website.

**About SGT-001**
Solid's SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy designed to address the underlying genetic cause of Duchenne muscular dystrophy (Duchenne). Duchenne is caused by mutations in the dystrophin gene that result in the absence or near absence of dystrophin protein. SGT-001 is a systemically administered candidate that delivers a synthetic dystrophin gene, called microdystrophin, to the body. This microdystrophin encodes for a functional protein surrogate that is expressed in muscles and stabilizes essential associated proteins, including neuronal nitric oxide synthase (nNOS). Data from Solid's preclinical program suggests that SGT-001 has the potential to slow or stop the progression of Duchenne, regardless of genetic mutation or disease stage.

SGT-001 is based on pioneering research in dystrophin biology by Dr. Jeffrey Chamberlain of the University of Washington and Dr. Dongsheng Duan of the University of Missouri. SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, in the United States and Orphan Drug Designations in both the United States and European Union.

**About Solid Biosciences**
Solid Biosciences is a life sciences company focused on developing transformative treatments to improve the lives of patients living with Duchenne muscular dystrophy (Duchenne). Disease-focused and founded by a family directly impacted by Duchenne, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation that causes Duchenne with our lead gene therapy candidate, SGT-001. For more information, please visit www.solidbio.com.

**Forward-Looking Statements**

This press release 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, the safety or potential efficacy of SGT-001 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. 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.

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