Solid Biosciences Provides Data Update from SGT-001 Development Program

December 18, 2019

- Demonstrated expression of SGT-001 microdystrophin and nNOS function provide evidence that SGT-001 has the potential to provide therapeutic benefit for patients with Duchenne

- Previously reported event observed in the third patient in the 2E14 vg/kg dose group has fully resolved

-Company received FDA clinical hold letter and is working to respond and determine a path to resume dosing

-Company to hold conference call at 8:30 AM ET today

CAMBRIDGE, Mass., Dec. 18, 2019 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) today announced biomarker data from two patients dosed in the second cohort of IGNITE DMD, its Phase I/II study of SGT-001. SGT-001 is the company's gene transfer candidate under investigation for Duchenne muscular dystrophy (Duchenne). The data from these patients show SGT-001 microdystrophin expression and associated neuronal nitric oxide synthase (nNOS) function, providing evidence that SGT-001 has the potential to result in therapeutic benefit for patients with Duchenne. The company also announced that the previously reported serious adverse event experienced by the third patient in the 2E14 vg/kg dose group has fully resolved, and the patient has resumed his normal activities. The company has received the clinical hold letter from the U.S. Food and Drug Administration (FDA) and will continue working internally, and with the FDA and other external experts, to address the clinical hold and determine the path forward.

"We now have evidence that SGT-001 can lead to microdystrophin expression at levels that we believe are meaningful and warrant further clinical development," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "I'm also pleased to say that the patient who experienced the event announced in November is doing well. We are steadfast in our commitment to bringing a transformative and safe therapy to the Duchenne community and are working diligently to resolve the clinical hold and resume dosing with SGT-001."

Six patients have been dosed with SGT-001 as part of IGNITE DMD; three at the 5E13 vg/kg dose and three at the 2E14 vg/kg dose. Three-month biopsies were recently analyzed from the fourth and fifth patients, both administered SGT-001 at 2E14 vg/kg. Using immunofluorescence assays, 10%-20% of microdystrophin positive muscle fibers were determined to express SGT-001 microdystrophin in the fourth patient and 50%-70% microdystrophin positive fibers in the fifth patient. Immunofluorescence also showed clear stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin in both patients. Inclusion of this nNOS coding region of the dystrophin protein may result in microdystrophin protein that has unique activity, potentially providing important functional benefits such as diminished muscle fatigue and protection against ischemic muscle damage. Using western blot, the expression levels for the fourth patient were detectable and estimated to be near the assay's level of quantification which is 5% of non-dystrophic control samples, with one assay replicate at 5.5%. Expression for the fifth patient was 17.5% of normal control samples. The levels of serum creatine kinase, a highly variable biochemical marker of muscle damage, declined from baseline in both patients. Collectively, these data provide evidence supporting the biological activity of SGT-001.

"SGT-001 represents the most advanced dystrophin biology in development for Duchenne," said Jeffrey Chamberlain, Professor of Neurology and McCaw Endowed Chair in Muscular Dystrophy at the University of Washington School of Medicine. "SGT-001's novel construct was specifically selected to drive expression of the drug's unique microdystrophin in cardiac and skeletal muscle, critical not only to mobility, but also to cardiac and pulmonary function. Continued evaluation of SGT-001 is essential to determine the ultimate clinical benefits that SGT-001 may provide for patients."

Conference Call Information
The company will host a conference call and webcast at 8:30 a.m. ET today to discuss the program update. Participants are invited to listen by dialing +1 866-763-0341 (domestic) or +1 703-871-3818 (international) five minutes prior to the start of the call and providing the passcode 5371089. A listen-only webcast of the conference call can also be accessed through the "Investors" tab on the Solid Biosciences website, www.solidbio.com, and a replay of the call will be available for approximately six weeks after the call.

About SGT-001
Solid's lead candidate, SGT-001, is a novel adeno-associated viral (AAV) vector-mediated gene transfer under investigation for its ability to address the underlying genetic cause of Duchenne, 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 science company focused solely on finding meaningful therapies for Duchenne muscular dystrophy (Duchenne). Founded by those touched by the disease, Solid is a center of excellence for Duchenne, bringing together experts in science, technology and care to drive forward a portfolio of candidates that have life-changing potential. Currently, Solid is progressing programs across four scientific platforms: Corrective Therapies, Disease-Modifying Therapies, Disease Understanding and Assistive Devices. 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. These risks and uncertainties include, but are not limited to, risks associated with Solid's ability to satisfactorily respond to requests from the FDA for further information and data regarding IGNITE DMD; successfully resolve the clinical hold with regard to IGNITE DMD; obtain and maintain necessary approvals from the FDA and other regulatory authorities and investigational review boards at clinical trial sites; enroll patients in its clinical trials; continue to advance SGT-001 in clinical trials; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully 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 DMD/Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. 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.

Investor Contact:
Carlo Tanzi, Ph.D.
Kendall Investor Relations
617-337-4680
investors@solidbio.com

Media Contact:
Courtney Heath
ScientPR
416-301-7966
media@solidbio.com

SOLID BIOSCIENCES

Source: Solid Biosciences Inc.