Solid Biosciences Announces Preliminary SGT-001 Data and Intention to Dose Escalate in IGNITE DMD Clinical Trial for Duchenne Muscular Dystrophy

February 7, 2019

- Company expediting planned dose escalation activities -

- Low levels of SGT-001 microdystrophin protein detected at the starting dose -

- Conference call to be held at 8:30 a.m. ET today -

CAMBRIDGE, Mass., Feb. 07, 2019 (GLOBE NEWSWIRE) -- Solid Biosciences (Nasdaq: SLDB) announced today preliminary findings from IGNITE DMD, the Company's Phase I/II dose-ascending clinical trial evaluating the safety and efficacy of SGT-001 microdystrophin gene transfer for the treatment of Duchenne muscular dystrophy (DMD). Initial three-month biopsy data showed low levels of microdystrophin protein expression. The Company is currently engaging with the appropriate parties to dose escalate as planned and as soon as possible.

"We believe that SGT-001 will be a meaningful treatment for patients with DMD and are confident we have the right approach in place to evaluate its potential at higher doses. We have already begun working to expedite the planned dose escalation strategy outlined in our clinical trial protocol," said Ilan Ganot, Co-founder, Chief Executive Officer and President of Solid Biosciences. "This strategy is further supported by our scalable manufacturing process, from which we have sufficient drug product available to dose escalate without delay. We have the financial resources to execute on our plan and look forward to communicating additional data later this year."

Preliminary analyses are based on three-month biopsy data from the first three patients dosed with 5E13 vg/kg of SGT-001, the lowest dose outlined in the study protocol. In one patient, microdystrophin was detected via western blot below the five percent level of quantification of the assay and in approximately 10 percent of fibers via immunofluorescence. There were also signs of co-localization of neuronal nitric oxide synthase (nNOS) and beta-sarcoglycan associated with microdystrophin expression. In the second and third patients, microdystrophin was detected via immunofluorescence at very low levels, but it was undetectable via western blot.

Six patients have been enrolled in IGNITE DMD, three to the active treatment group and three to the delayed treatment control group. The safety profile of SGT-001 remains unchanged and all patients continue to be followed per the study protocol.

"The patients who have received SGT-001 as part of the IGNITE DMD clinical trial are all doing well, and we are encouraged to explore higher doses moving forward," said Dr. Barry Byrne, M.D., Ph.D., Professor of Pediatrics and principal investigator for IGNITE DMD. "It is extremely important to advance innovative research with the ultimate goal to bring therapies to patients with Duchenne muscular dystrophy."

As previously disclosed, Solid believes that its existing cash, cash equivalents and available-for-sale securities as of September 30, 2018 will be sufficient to fund its operations through the first quarter of 2020.

Conference Call:

The company will host a conference call and webcast at 8:30 a.m. ET today to discuss these preliminary findings. 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 2153338. 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 there for six weeks after the call.

About IGNITE DMD:

IGNITE DMD is a randomized, controlled, open-label, single-ascending dose Phase I/II clinical trial to evaluate SGT-001 in ambulatory and non-ambulatory males with DMD aged 4 to 17 years. The primary objectives of the study are to assess the safety and tolerability of SGT-001, as well as efficacy as defined by microdystrophin expression. The study will also assess muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with microdystrophin production, patient reported outcomes and quality of life measures, among other endpoints. Participants will be randomly assigned to either an active treatment group or a delayed treatment control group. Participants in the control group who continue to meet the study's treatment criteria will receive active treatment after 12 months.

More information on IGNITE DMD can be found on www.clinicaltrials.gov under identifier number NCT03368742.

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 muscular dystrophy (DMD), 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 transgene, 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). SGT-001 utilizes AAV9, which has an affinity for muscle and is currently being evaluated in multiple clinical programs in other indications. Data from Solid's preclinical program suggest that SGT-001 has the potential to slow or stop the progression of DMD, 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, and Fast Track Designation 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 (DMD). Founded by those touched by the disease, Solid is a center of excellence for DMD, bringing together experts in science, technology and care to drive forward a portfolio of candidates that have life-changing potential. 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 Solid’s IGNITE DMD clinical trial, and the potential of SGT-001. 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 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, including to proceed with dose escalation of IGNITE DMD; 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; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop DMD 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.

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