Solid Biosciences Provides SGT-001 Program Update

November 12, 2019

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

CAMBRIDGE, Mass., Nov. 12, 2019 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) today provided a clinical update on SGT-001 and reported that the U.S. Food and Drug Administration (FDA) has notified the company that IGNITE DMD, its Phase I/II study of SGT-001, has been placed on clinical hold. The company will hold a webcast conference call this morning to discuss this update.

To date, six patients have been dosed with SGT-001, Solid's gene transfer candidate under investigation for Duchenne muscular dystrophy (DMD). This includes three patients in the first cohort at a 5E13 vg/kg dose, who continue to do well and are being followed per the study protocol. Three patients were subsequently dosed in the second cohort at a 2E14 vg/kg dose. The first two of these patients are also doing well and being followed per study protocol.

The third patient in the 2E14 vg/kg cohort, dosed in late October, experienced a serious adverse event (SAE) deemed related to the study drug that was characterized by complement activation, thrombocytopenia, a decrease in red blood cell count, acute kidney injury, and cardio-pulmonary insufficiency. Neither cytokine- nor coagulopathy-related abnormalities were observed. Currently the patient is being closely followed by his care team. He is recovering and continues to improve.

The company reported the event to the FDA and the study Data Safety Monitoring Board (DSMB). The FDA has notified the company that the study has been placed on clinical hold. Solid will work with the FDA in an effort to resolve the hold and determine next steps for IGNITE DMD. The company continues to plan to report additional biomarker data from the study before year end.

"We are encouraged that this patient is recovering. I would like to thank both the patient and his family for their participation in our study, as well as the team at the University of Florida for the excellent care they provide," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "We remain committed to bringing meaningful new therapies to the Duchenne community and continue to believe in the differentiated construct of SGT-001 and the potential benefits it may offer to patients. In the coming weeks, we anticipate that we will have a better understanding of the biological activity and potential benefit of SGT-001. We look forward to sharing this additional data and working with the FDA to resolve the clinical hold and determining next steps for the program."

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 2277849. 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 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 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 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, 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. 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 Solid's intentions regarding communications with the FDA and its 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," 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 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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