Solid Biosciences Provides Update Regarding the IGNITE DMD Phase I/II Clinical Hold

July 24, 2020

CAMBRIDGE, Mass., July 24, 2020 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy, today announced that it received written communication from the U.S. Food and Drug Administration (FDA) that the Company's IGNITE DMD Phase I/II clinical trial remains on clinical hold.

In November 2019, the FDA placed IGNITE DMD on clinical hold as the result of a serious adverse event (SAE) that occurred in the sixth patient dosed in the trial; as previously reported the SAE has fully resolved. In April 2020, Solid provided the FDA with information and measures intended to improve patient safety and in May 2020, Solid received written communication from the FDA that the trial remained on hold. In June 2020, the Company submitted a response to the FDA that provided data related to manufacturing process improvements. Today, the FDA responded by maintaining the clinical hold and requesting further manufacturing information, updated safety and efficacy data for all patients dosed, and providing direction on total viral load to be administered per patient. Solid is evaluating next steps and will provide an update as appropriate.

"Patient safety is our highest priority and we plan to continue our dialogue with the FDA to determine the appropriate path for SGT-001," said Ilan Ganot, Co-Founder, President and Chief Executive Officer of Solid Biosciences. "We are disappointed with the outcome today, however, we will continue to work with the FDA as we believe the clinical development of SGT-001 could offer meaningful benefits to patients with this devastating disease."

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 Company's expected dialogue with the FDA regarding the clinical hold, the potential lifting of the clinical hold and 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 the Company'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 on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities and investigational review boards at clinical trial sites; enroll patients in IGNITE DMD; 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.

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