Solid Biosciences Provides Update regarding SGT-001 Phase I/II Clinical Hold on IGNITE DMD

May 7, 2020

CAMBRIDGE, Mass., May 07, 2020 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy, announced that it received written communication from the U.S. Food and Drug Administration (FDA) regarding the clinical hold placed on the Company's IGNITE DMD Phase I/II clinical trial. The program remains on clinical hold and the Company will continue to work with the FDA to address their requests.

In April 2020, the Company submitted a response to the FDA that included changes to the clinical protocol designed to enhance patient safety, as well as information related to improvements to its manufacturing process. The FDA has responded by maintaining the clinical hold and requesting further data and analyses relating to this manufacturing process. The Company is in the process of generating these data and expects to submit this information to the FDA before the end of the third quarter of 2020.

"We share the FDA's commitment to patient safety and are working collaboratively with the agency to resolve the clinical hold," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "We consider patient safety the utmost priority and believe the clinical development of SGT-001 could offer meaningful benefits to patients with this devastating disease."

In addition, Solid Biosciences today filed its **Quarterly Report on Form 10-Q** for the quarter ended March 31, 2020 with the Securities and Exchange Commission.

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 response to the FDA with respect to the clinical hold on the IGNITE DMD clinical trial, the potential lifting of the clinical hold on the Company's IGNITE DMD clinical trial, 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 on the timeline expected or at all; 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:
David Carey
FINN Partners
212-867-1768
David.Carey@finnpartners.com

Media Contact:
Erich Sandoval

FINN Partners
917-497-2867
Erich.Sandoval@finnpartners.com

Source: Solid Biosciences Inc.