

Solid Biosciences, LLC Logo

## Solid Biosciences Provides Update On SGT-001 Clinical Development Program For Duchenne Muscular Dystrophy

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- Full Clinical Hold Letter from the FDA Received; Company to Respond in the Coming Weeks -
- Manufacturing-related Partial Clinical Hold on the High Dose of SGT-001 Resolved -

CAMBRIDGE, Mass., April 18, 2018 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (NASDAQ:SLDB) today announced that the Company has received a letter from the U.S. Food and Drug Administration (FDA) relating to the previously-announced full clinical hold on IGNITE DMD, the Company's Phase I/II clinical trial for its investigational gene therapy, SGT-001, for the treatment of Duchenne muscular dystrophy (DMD). The Company plans to submit a response to this letter to the FDA in the coming weeks. The FDA also informed Solid that the Company has satisfactorily addressed manufacturing-related questions that led to the previously-announced partial clinical hold on the high dose of SGT-001 in IGNITE DMD.

"We believe SGT-001 has the potential to significantly benefit patients with DMD. We are working with the FDA to better understand the unexpected event that resulted in the clinical hold and put a plan in place to monitor and manage potential events in the future. I am happy to share that the treating physician has reported the patient is doing well," said Ilan Ganot, Chief Executive Officer of Solid Biosciences. "We are also pleased that we were able to lift the earlier partial clinical hold on the high dose of SGT-001 through our existing manufacturing process, which is an important ongoing development effort for our company."

The full clinical hold was in response to the Company's report of a Serious Adverse Event (SAE) in the first patient dosed with SGT-001 in IGNITE DMD, where several days after administration the patient was hospitalized due to laboratory findings that included a decrease in platelet count followed by a reduction in red blood cell count and evidence of complement activation. The patient showed no signs or symptoms of coagulopathy and no relevant changes from baseline in liver function tests. Following medical treatment, all of his laboratory parameters have either improved or returned to normal.

In its full clinical hold letter, the FDA requested additional information that is required prior to resuming the clinical trial, including an assessment of the underlying etiology of the event, the patient's clinical status and laboratory parameters, and any additional measures to address patient safety. Solid will work closely with the FDA to address these questions.

The earlier partial clinical hold on IGNITE DMD was related to manufacturing processes for the high dose of SGT-001. Solid submitted additional data to the FDA demonstrating that the current manufacturing process and product attributes for SGT-001 could support the high-dose group, enabling the use of a single lot for dose administration and limiting the number of vials of product required to treat each patient. The FDA notified Solid that its response has been accepted, and the partial clinical hold is now resolved.

### 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 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. 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](http://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 ongoing IGNITE DMD clinical trial. 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 later clinical trials positive results found in preclinical studies and earlier stage clinical trials of SGT-001 and its other product candidates; 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 our 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 our 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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