

Solid Biosciences, LLC Logo

## Solid Biosciences Announces FDA Removes Clinical Hold on SGT-001

June 18, 2018

*- Activities to Resume Enrollment in IGNITE DMD Phase I/II Clinical Trial are Underway*

*- Conference Call Scheduled for Today at 8:30 A.M. ET*

CAMBRIDGE, Mass., June 18, 2018 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (NASDAQ:SLDB) today announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold on IGNITE DMD, the Company's Phase I/II clinical trial for its investigational microdystrophin gene transfer, SGT-001, for the treatment of Duchenne muscular dystrophy (DMD). In its letter, the FDA acknowledged that the Company satisfactorily addressed all clinical hold questions. Solid has begun activities to resume the clinical trial and plans to reinitiate enrollment as quickly as possible.

"We believe SGT-001 has the potential to offer significant benefit to patients with DMD, regardless of their age or stage of disease," said Ilan Ganot, Founder and Chief Executive Officer of Solid Biosciences. "We are pleased to have been able to provide the FDA with a comprehensive response resulting in the removal of the clinical hold so we can continue development of this important potential treatment."

As previously disclosed, the FDA placed a clinical hold on IGNITE DMD following the Company's report of a Serious Adverse Event (SAE) in the first patient dosed with SGT-001. The event was characterized by a decrease in platelet count followed by a reduction in red blood cell count, transient renal impairment and evidence of complement activation. There were no signs of bleeding or clotting abnormalities and no laboratory evidence of liver dysfunction. The patient received standard medical care, a modified steroid regimen and a limited course of eculizumab for the observed complement activation. He remained clinically stable and generally asymptomatic throughout the event, which fully resolved.

"Gene therapy has the potential to dramatically change the course of DMD and may offer long-term benefit for those who suffer from this devastating disease," said Barry Byrne, M.D., Ph.D., Director, University of Florida Powell Gene Therapy Center and Professor, Pediatrics and Molecular Genetics & Microbiology at the University of Florida College of Medicine. "After a thorough analysis of the clinical and laboratory data for the patient, I am confident the event was easily monitored and medically manageable. Our patient quickly returned to his normal activities and planned study assessments. We look forward to continuing the IGNITE DMD study and providing additional children and adolescents with this promising investigational therapy."

In connection with the lifting of the clinical hold, Solid has made changes to the IGNITE DMD protocol, including the addition of IV glucocorticoids in the initial weeks post administration of SGT-001 and enhanced monitoring measures that include a panel for complement activation. The amended protocol also specifies that eculizumab will be available as a treatment option if complement activation is observed.

The Company plans to enroll and dose several children prior to dosing additional adolescents. In addition, Solid now has the choice to obtain the intermediate muscle biopsy at 45 days post administration of SGT-001 to collect additional information about the time course of microdystrophin expression.

As a result of the clinical hold, Solid now expects to report initial data from a pre-specified interim analysis of IGNITE DMD in the second half of 2019.

### Conference Call

Solid's management team will host a conference call and webcast at 8:30 a.m. ET today to discuss the lifting of the clinical hold. The conference call can be accessed by dialing +1 866 763 0341 for domestic callers and +1 703 871 3818 for international callers. The passcode for the call is 8892798. A live webcast of the conference call can also be accessed through the "Investors" tab on the Solid Biosciences website at [www.solidbio.com](http://www.solidbio.com). A webcast replay will be available online 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 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, in the United States and Orphan Drug Designations in both the United States and the 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 Statement

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 development; replicate in later 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 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 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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