

Solid Biosciences, LLC Logo

## **Solid Biosciences Announces Clinical Hold On SGT-001 Phase I/II Clinical Trial For Duchenne Muscular Dystrophy**

March 14, 2018

CAMBRIDGE, Mass., March 14, 2018 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (NASDAQ:SLDB) today announced it has received notification from the U.S. Food and Drug Administration (FDA) that IGNITE DMD, its Phase I/II clinical trial for SGT-001 microdystrophin gene transfer in Duchenne muscular dystrophy (DMD), has been placed on Clinical Hold.

IGNITE DMD is designed to assess the safety and efficacy of SGT-001 in ambulatory and non-ambulatory children and adolescents with DMD. The first patient dosed in the clinical trial was a non-ambulatory adolescent who received 5E13 vg/kg of SGT-001 on February 14, 2018. 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 (bleeding disorder) and no relevant changes from baseline in liver function tests. The patient responded well to medical treatment and is currently asymptomatic. All laboratory parameters have either improved or returned to normal, and he is continuing outpatient assessments per protocol.

Solid reported the event to the FDA and, because it was unexpected, classified it as a Suspected Unexpected Serious Adverse Reaction (SUSAR). The FDA informed the company that the clinical hold was due to the event. Solid has halted enrollment and dosing in IGNITE DMD and is awaiting the formal Clinical Hold letter from the FDA to understand the requirements for resuming the clinical trial. Solid will work closely with the Agency to resolve the Clinical Hold.

### **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](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 and the partial clinical hold with respect to the high dose of SGT-001; 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.

### **Contact:**

Solid Biosciences  
Kate Niazi-Sai  
617-337-4680  
[media@solidbio.com](mailto:media@solidbio.com)  
[investors@solidbio.com](mailto:investors@solidbio.com)

Source: Solid Biosciences Inc.