Solid Biosciences, LLC Logo

Solid Biosciences Initiates Clinical Trial for Gene Transfer Candidate SGT-001 in Patients with Duchenne Muscular Dystrophy

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-Phase I/II adaptive clinical trial to evaluate safety and efficacy in ambulatory and non-ambulatory children and adolescents-

-Interim analysis anticipated in the first half of 2019-

Cambridge, MA, November 30, 2017 -- Solid Biosciences announced today that it has initiated clinical trial activities for SGT-001, the company's lead microdystrophin gene transfer candidate for the treatment of Duchenne muscular dystrophy (DMD). The Phase I/II adaptive study, called IGNITE DMD, will evaluate the safety and efficacy of a single intravenous (IV) dose of SGT-001 in ambulatory and non-ambulatory adolescents and children with DMD. Enrollment will begin at the first clinical trial site in the United States in the coming days.

> "For more than three years, we've been working on a preclinical package and scalable manufacturing process for SGT-001 that would allow us to responsibly move into the clinic," said Ilan Ganot, Founder and Chief Executive Officer of Solid Biosciences. "This work, as well as decades of research by our scientific advisors and insight from the DMD community, helped us design a clinical trial that will efficiently characterize the safety and efficacy of SGT-001 in both ambulatory and non-ambulatory patients, regardless of their underlying genetic mutation."

IGNITE DMD, a single-ascending dose study, is adaptive in nature, which will allow Solid to adjust the dose and number of participants as the study progresses to efficiently assess the safety and efficacy of SGT-001. Solid expects to enroll approximately 16 to 32 patients in the study, all of whom will receive a systemic dose of SGT-001. The starting dose was selected based on Solid's extensive preclinical program across multiple animal species. All clinical drug product is being produced utilizing Solid's scalable manufacturing process.

Solid is initiating its study under an Investigational New Drug (IND) application cleared by the U.S. Food and Drug Administration (FDA) on October 12, 2017. A pre-specified interim analysis of preliminary data is planned and will be communicated in the first half of 2019.

"For more than 20 years, my team has focused our research on understanding the potential of microdystrophin in DMD and optimizing a construct that we believe will provide the most benefit for patients," said Dr. Jeffrey Chamberlain, Professor, Department of Neurology; Professor, Department of Biochemistry; and Professor of Medicine, Division of Medical Genetics at the University of Washington. "I'm pleased to see Solid advance the most compelling of constructs into clinical studies. I believe we are now at an inflection point where, if successful, SGT-001 should represent an innovative new way of treating patients with this devastating disease."

About IGNITE DMD:

IGNITE DMD is a randomized, controlled, open-label, single-ascending dose Phase I/II clinical study to evaluate SGT-001 in ambulatory and non-ambulatory males with DMD aged 4 to 17 years. The primary objectives of the study are to assess the safety and tolerability of SGT-001, as well as efficacy as defined by microdystrophin expression. The study will also assess muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with microdystrophin production, patient reported outcomes and quality of life measures, among other endpoints. Participants will be randomly assigned to either an active treatment group or a delayed treatment control group. Participants in the control group who continue to meet the study's treatment criteria will receive active treatment after 12 months.

The IGNITE DMD study will be initially conducted at clinical trial sites in the United States. More information on the study will be posted on www.clinicaltrials.gov in the coming days.

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 robust 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. Dongsheng Duan of the University of Missouri and Dr. Jeffrey Chamberlain of the University of Washington. 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. The candidate is currently being evaluated in a Phase I/II clinical study.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, genetic muscle-wasting disease that is progressive, irreversible and ultimately fatal. DMD affects approximately one in every 3,500 to 5,000 live male births. DMD is caused by mutations in the dystrophin gene that result in the absence or near-absence of dystrophin, a protein critical for muscle health. Without functioning dystrophin, muscles in the body suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of scar and fat tissue.

Symptoms of DMD usually manifest between three to five years of age. As the disease progresses, patients typically lose the ability to walk by their early teens and succumb to respiratory or heart failure in early adulthood. There is no cure for DMD and, for the vast majority of patients, there are no satisfactory treatments.

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.

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