Solid Biosciences, LLC Logo

Solid Biosciences Granted U.S. and E.U. Orphan Drug Designations for Lead Gene Therapy Candidate for Duchenne Muscular Dystrophy

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Company Expands Presence with London Office to Support Clinical Development.

Solid Biosciences and its subsidiary, Solid GT, announced today that the U.S. Food and Drug Administration and the European Commission have granted Orphan Drug designations for the company's gene therapy candidate, SGT-001, for the treatment of patients with Duchenne muscular dystrophy (DMD). Solid plans to initiate clinical studies for SGT-001 in 2017.

To support the advancement of its clinical program, Solid has opened an office in London, England. Spearheaded by leading DMD clinician Valeria Ricotti, M.D., Ph.D., (formerly University College London, Institute of Child Health and Great Ormond Street Hospital) and patient advocate Kerry Rosenfeld, Solid's London team will work closely with the European DMD community to help realize its goal of bringing meaningful therapies to patients around the world.

> "The orphan designations mark a positive step forward in our efforts to advance SGT-001 through development and to patients with DMD in both the United States and Europe," said Ilan Ganot, chief executive officer and founder of Solid Biosciences. "Our teams in the United States and in our new offices in London are working tirelessly to bring our gene therapy candidate SGT-001 to patients as soon as possible."

Duchenne muscular dystrophy is a rare, progressive muscle-wasting disease that affects approximately 1 in 3,500-5,000 boys born worldwide and is the most common fatal genetic disorder diagnosed in childhood. It is caused by the absence of the dystrophin protein, the fundamental mediator of skeletal and cardiac muscle function[i]. SGT-001 is an adeno-associated viral (AAV) vector-mediated gene therapy candidate that is being developed for its potential to restore functional dystrophin expression in muscle.

In preclinical studies, a single administration of SGT-001 resulted in long-term, systemic expression of micro-dystrophin, a shorter form of dystrophin, in muscle. SGT-001 also improved function, as measured by improved muscle strength and protection against contraction-induced damage.

Orphan Drug designations offer companies incentives and support for developing therapies for rare diseases. In the U.S., the FDA's Office of Orphan Products Development grants Orphan Drug Designation to therapies intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States[ii]. In the European Union, the European Medicines Agency (EMA) renders an opinion on Orphan designation applications for candidates intended for the treatment of life-threatening or chronically debilitating rare conditions with a prevalence of less than five in 10,000 in the European Union.[iii] The European Commission then adopts the EMA's opinion through a separate approval process.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, muscle-wasting disease. As the most common fatal genetic disorder diagnosed in childhood, it affects approximately 1 in 3,500-5,000 boys born worldwide, yet there is a significant need for treatments that can benefit all boys with the disease. DMD is caused by the absence of dystrophin, a protein that is fundamental for muscle function. Because of the lack of dystrophin, patients experience progressive and pervasive muscular degeneration, which eventually results in premature death. Patients are typically wheelchair-bound by their early teens and succumb to respiratory or heart failure in their 30s.

About Solid Biosciences

Solid Biosciences is a life science company focused solely on finding meaningful therapies for Duchenne muscular dystrophy (DMD). Founded by those directly impacted by the disease, Solid aims to be the center of excellence across the entire DMD disease spectrum, 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.

About Solid GT and SGT-001

Solid GT, a subsidiary of Solid Biosciences, is focused on developing novel, genetic interventions that have the potential to slow or even halt the progression of Duchenne muscular dystrophy (DMD). Solid GT's lead candidate, SGT-001, is an adeno-associated viral (AAV) vector-mediated gene therapy. SGT-001 is being investigated in IND-enabling studies for its potential to drive the expression of micro-dystrophin, a shorter form of the dystrophin protein that is fundamental for muscle function yet is missing in patients with DMD. Solid GT anticipates initiating clinical studies for SGT-001 in 2017. For more information, please visit www.solidbio.com/GT.

[i] Parent Project Muscular Dystrophy. About Duchenne. http://www.parentprojectmd.org/site/PageServer?pagename=Understand_about. Accessed August 2016.

[ii] US Food and Drug Administration. Developing Products for Rare Diseases & Conditions. http://www.fda.gov/ForIndustry

/DevelopingProductsforRareDiseasesConditions/default.htm. Accessed August 2016.

[iii] European Medicines Agency. Orphan designation. http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp. Accessed October 2016.