Solid Biosciences, LLC Logo

New Preclinical Data Support SGT-001 As A Novel Treatment Approach For Duchenne Muscular Dystrophy

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Solid Biosciences to Initiate Clinical Program for Gene Therapy Candidate in Coming Months

Cambridge, MA – May 11, 2017 – Solid Biosciences announced today that new data from two preclinical studies reinforce the potential of its investigational microdystrophin gene therapy, SGT-001, to be an effective treatment approach for Duchenne muscular dystrophy (DMD). The preclinical data, which were presented at the American Society of Gene and Cell Therapy (ASGCT) 20th Annual Meeting, demonstrated that a single administration of SGT-001 resulted in sustained and significant microdystrophin expression and improvements in muscle function, with no observed immune response. The Company plans to initiate clinical trials for SGT-001 in the second half of 2017.

> “These preclinical data show the potential efficacy, durability and tolerability of SGT-001 and give us further confidence in our plan to initiate our clinical program later this year,” said Joel Schneider, Ph.D., vice president of Research and Development at Solid Biosciences. “I would like to thank our academic partners for their work to help us build one of the most robust gene therapy preclinical datasets in DMD and for sharing our mission to bring meaningful treatments to patients with this devastating disease.”

Data from the studies were presented by Solid’s collaborators at the University of Missouri and Texas A&M University in two posters:

- Results from an ongoing, long-term, dose-ranging preclinical study in canines showed that systemic administration of four different doses of SGT-001 led to body-wide transgene expression, which was dose-dependent and sustained for up to 24 months. This expression correlated with improvements in multiple functional parameters.
- Results from a blinded, dose-ranging preclinical study in canines showed that three different doses of SGT-001 led to statistically significant dose-dependent improvements in muscle function compared to non-treated animals. These improvements correlated with sustained transgene expression during the study period.

In both studies, administration of SGT-001 was well-tolerated and there was no observed immune response to the transgene.

To complement these findings, Solid also presented data to validate the utility of several seroprevalence assays in the preclinical setting. All of the data are part of Solid’s IND-enabling preclinical program for SGT-001. The safety and efficacy of SGT-001 will be further evaluated in clinical trials.

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 one 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 early adulthood.

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 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. The Company’s lead candidate, SGT-001, is an adeno-associated viral (AAV) vector-mediated gene therapy for which clinical trials are anticipated to begin in the second half of 2017. For more information, please visit www.solidbio.com.