Solid Biosciences, LLC Logo

Solid Biosciences Announces New Preclinical Data at the American Society of Gene and Cell Therapy Annual Meeting

May 18, 2018

- Data Reinforce Potential of SGT-001 as an Important Treatment Candidate for DMD -

- Company Continues to Advance Gene Therapy Portfolio -

CAMBRIDGE, Mass., May 18, 2018 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (NASDAQ:SLDB) today announced the presentation of new preclinical data from its gene therapy development programs for Duchenne muscular dystrophy (DMD). New data for SGT-001, the Company's lead microdystrophin gene transfer candidate, further demonstrate its potential to produce long-term and body-wide microdystrophin expression that correlates with significant improvements in muscle function. Additionally, the Company presented data supporting the development of novel promoters and capsids as part of its next generation gene therapy discovery efforts. These data were presented this week at the 21st Annual Meeting of The American Society of Gene and Cell Therapy (ASGCT) in Chicago.

"Solid remains steadfast in our mission to bring meaningful therapies to patients with DMD, where significant unmet need exists. These data further support the investigation of SGT-001 as a potential new treatment option for those living with this devastating disease, as well as reinforce our commitment to advancing cutting-edge innovations through our next generation gene therapy pipeline," said Carl Morris, Ph.D., Chief Scientific Officer of Solid Biosciences. "This focus on rigorous science is reflected in our growing, multifaceted pipeline of candidates aimed at addressing all of the manifestations of the disease."

Data Highlights from the SGT-001 Preclinical Program
New preclinical data help further characterize the potential efficacy profile of SGT-001.

AAV Micro-Dystrophin Therapy Ameliorates Muscular Dystrophy in Young Adult Duchenne Muscular Dystrophy Dogs for Up to Thirty Months Following Injection
Oral presentation: abstract #8

Long-term preclinical data in canines demonstrated that a single intravenous administration of a low, medium or high dose of SGT-001 resulted in body-wide microdystrophin transgene expression, as well as restoration of key microdystrophin-associated proteins, including neuronal nitric oxide synthase (nNOS). This expression, which was sustained for at least 30 months, was associated with reduced muscle pathology and improved muscle function. These data were presented by Solid's collaborators at the University of Missouri.

In Vivo Comparison of the Biological Potency of rAAV9-Microdystrophin Made by Transient Transfection and a Scalable Herpesvirus System
Poster presentation: abstract #626

Additional preclinical data presented by the University of Missouri demonstrated that AAV microdystrophin vector produced by the herpesvirus system is comparable to that made by transient transfection in ameliorating muscle disease in the mouse model.

Preclinical Evaluation of SGT-001 Microdystrophin Gene Transfer for Duchenne Muscular Dystrophy
Poster presentation: abstract #854

New preclinical data evaluating the relationship of SGT-001 and nNOS demonstrated that a dose-dependent increase in nNOS protein expression and associated nNOS activity corresponded with microdystrophin expression. This resulted in improvements in muscle histopathology and muscle function in both small and large animal models, suggesting nNOS activity could serve as a marker of molecular function.

Assessing Anti-Dystrophin T-Cell Responses by Elispot Following AAV9-Microdystrophin Gene Therapy in Dogs
Poster presentation: abstract #219

A preclinical study conducted by Solid's collaborators at the University of Washington School of Medicine showed that no T-cell response was detected or induced by the SGT-001 microdystrophin transgene in canines at least three months after administration.

Complementary Techniques to Evaluate Microdystrophin Expression in Duchenne Muscular Dystrophy Gene Therapy Studies
Poster presentation: abstract #868

New data presented by the Medical College of Wisconsin suggest that a combination of both quantitative and qualitative assays (Western Blot, Mass Spectrometry and Immunofluorescence) could support the reliable detection of microdystrophin expression after SGT-001 administration.

Data Highlights from Solid's Next Generation Gene Therapy Pipeline
New data from Solid's exploratory gene therapy pipeline support the Company's efforts to develop the next generation of gene therapies to increase efficacy, as well as to address re-administration and neutralizing antibodies.

Identification of Novel AAV Capsids for Skeletal Muscle Gene Transfer by In Vivo Selection in Humanized Mice
Oral presentation: abstract #355

New data presented by Solid's collaborators at the University of Massachusetts Medical School showed that several novel capsids identified through DNA shuffling demonstrated muscle tropism and expression similar to that of AAV9, one of which exhibited decreased transduction of the liver, spleen and lung.

In Silico Platform for the Design and Generation of Novel Muscle Promoters: In Vitro Validation
Poster presentation: abstract #869

New data from Solid's collaboration with Synpromics Ltd demonstrated that a novel set of key muscle-selective promoters have the potential to enhance transgene expression, size and tissue selectivity.

Abstracts for all data presented can be accessed at https://plan.core-apps.com/asgct2018/abstracts.

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 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 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. The Phase I/II clinical trial for SGT-001, IGNITE DMD, is currently on clinical hold.

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.

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 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 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; 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.

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