Solid Biosciences Announces Publication Highlighting the Important Role of the nNOS Domain in Microdystrophin Function and its Potential Utility in DMD Gene Therapy in the Journal Neuromuscular Disorders

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CAMBRIDGE, Mass., Sept. 05, 2019 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) today announced the publication of a review article in the journal *Neuromuscular Disorders*[1] that summarizes the evidence supporting the function of microdystrophin-associated neuronal nitric oxide synthase (nNOS) and its potential utility in Duchenne muscular dystrophy (DMD) gene therapy.

The publication reviews the available preclinical data, as well as human data from individuals with Becker muscular dystrophy (BMD), summarizing the evidence linking nNOS activity and proper cell localization to its role in counteracting muscle ischemia and in maintaining muscle health. Clinical evidence based on individuals with BMD, a less severe form of muscular dystrophy than DMD, indicates that deficiency in nNOS activity is associated with more severe disease progression.

“Dystrophin is a large and complex protein with multiple biological activities,” said Carl Morris, Ph.D., senior study author and Chief Scientific Officer at Solid Biosciences. “Evidence indicates that the nNOS domain is a critical component of dystrophin and may be required for normal protein function. Our AAV9-based microdystrophin gene therapy development candidate, SGT-001, is unique in that it includes the functional nNOS domain, and our development program will assess potential nNOS-related clinical benefits, including diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle. We look forward to continuing to evaluate the clinical profile of SGT-001 in the ongoing Phase 1/2 IGNITE DMD trial in patients with DMD.”

[1] The publication, *Membrane recruitment of nNOSμ in microdystrophin gene transfer to enhance durability*, is available online on the Neuromuscular Disorders website: https://www.nmd-journal.com/article/S0960-8966(19)31100-9/fulltext

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 Duchenne muscular dystrophy (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 suggest 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, and Fast Track Designation in the United States and Orphan Drug Designations in both the United States and the 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. 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 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 IGNITE DMD clinical trial, the potential of SGT-001 and nNOS protein activity. 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 continue to advance SGT-001 in clinical trials; replicate in later clinical trials positive results found in preclinical studies and earlier stages of clinical development; and advance the development of its product candidates under the timelines it anticipates in current and future clinical trials. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company’s 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 the Company’s 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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