Solid Biosciences Reports Additional Pulmonary Function Results from the Ongoing IGNITE DMD Phase I/II Clinical Trial of SGT-001

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- Improvements in two additional assessments of pulmonary function in treated patients compared with untreated control patients further support potential functional benefit of SGT-001 one-year post administration -

- Data presented at the Child Neurology Society 50th Annual Meeting -

CAMBRIDGE, Mass., Sept. 29, 2021 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today reported additional positive pulmonary function data from the first six patients in the ongoing IGNITE DMD Phase I/II clinical trial of SGT-001. Oscar H. Mayer, MD, Attending Pulmonologist and Director of the Pulmonary Function Laboratory at Children's Hospital of Philadelphia, is presenting the data in a poster session (Abstract #78) at the Child Neurology Society 50th Annual Meeting.

"Duchenne is ultimately a fatal disease, with patients often succumbing to cardiopulmonary failure as muscle cells in the diaphragm and heart deteriorate," said Roxana Donisa Dreghici, Senior Vice President, Head of Clinical Development for Solid Biosciences. "Consequently, the ability to improve pulmonary function in these patients, especially during a period when the untreated control cohort and natural history data indicate functional decline, is evidence of the potentially meaningful clinical benefit of SGT-001."

"The improvements in pulmonary function endpoints seen in the IGNITE DMD study, from baseline to one year are very promising, especially given that loss of pulmonary function leads to respiratory failure and ultimately death and, to varying degrees, impacts all patients living with Duchenne muscular dystrophy," said Dr. Mayer. "The potential to improve, stabilize or even slow the decline in pulmonary function would be clinically beneficial and meaningful to patients. Continued collection and analysis of pulmonary function data in IGNITE DMD should provide additional insight into the potential benefit that SGT-001 may provide for patients with Duchenne muscular dystrophy."

IGNITE DMD Pulmonary Function Data
The pulmonary function results include percent predicted peak expiratory flow (PEF % predicted) and forced expiratory volume in one second (FEV1 % predicted) one year post SGT-001 administration. The data were collected from the first six patients dosed in IGNITE DMD (three at the low dose of 5E13 vg/kg and three at the high dose of 2E14 vg/kg) and three untreated control patients. Certain patients were not evaluable due to either missed patient assessments or not meeting the clinically acceptable criteria for the respective assessment.

- Four of these six patients dosed with SGT-001 and two of three patients in the untreated control cohort were evaluable for PEF % predicted assessment, with improvements noted in all dosed patients and declines noted in the untreated control patients. Patients in the low-dose cohort, Patients 1 and 3, demonstrated improvements in PEF % predicted from baseline to 1 year of 2.5% and 38.5%, respectively, and patients in the high-dose cohort, Patients 4 and 6, demonstrated improvements of 15.9% and 26.7%, respectively, at 1 year. Patients in the untreated control cohort, Control Patients 1 and 3 had declines of 1.1% and 18.2%, respectively, at 1 year.

- Five of these six patients dosed with SGT-001 and all three patients in the untreated control cohort were evaluable for FEV1 % predicted assessment, with improvements noted in all dosed patients and declines noted in the untreated control patients. Patients in the low-dose cohort, Patients 1 and 3, demonstrated improvements in FEV1 % predicted from baseline to 1 year of 13.4% and 4.3%, respectively, and patients in the high-dose cohort, Patients 4-6, demonstrated improvements of 10.8%, 15.5% and 2.8%, respectively, at 1 year. Patients in the untreated control cohort, Control Patients 1-3, reported declines of 8.7%, 17.0% and 12.0%, respectively, at 1 year.

These data, together with another pulmonary function endpoint, percent predicted forced vital capacity (FVC), provide further evidence that SGT-001 may benefit patients with respect to pulmonary function. Earlier this month, Solid reported positive 1.5-year data showing improvements in FVC at the World Muscle Society 2021 Virtual Congress, along with 1.5-year data from other functional and patient-reported outcome measures. The mean improvement in percent predicted FVC from baseline to 1.5 years for Patients 4-6 was 8.5% (Range: +0.6% to +22.5%), and the mean difference compared with natural history data was +16.0% over the same time period.

Natural history analyses of pulmonary function data suggest that patients would normally expect to exhibit a decline over the same time periods assessed.

“To our knowledge, Solid is the first company to report improvement in multiple assessments of pulmonary function following administration of a Duchenne gene therapy,” said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. “These data add to the data we have previously reported from the IGNITE DMD clinical trial. We believe that exploring diverse endpoints will enable us to better understand the totality of the potential benefits that SGT-001 may provide across the spectrum of Duchenne-related disease manifestations.”

About SGT-001
Solid’s SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy designed to address the underlying genetic cause of Duchenne. Duchenne is caused by 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 clinical program suggests that SGT-001 has the potential to slow or stop the progression of Duchenne, 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 European Union.

About Solid Biosciences
Solid Biosciences is a life sciences company focused on advancing transformative treatments to improve the lives of patients living with Duchenne. Disease-focused and founded by a family directly impacted by Duchenne, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation that causes Duchenne with our lead gene therapy candidate, SGT-001. 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 the ability of the Company to continue dosing patients in the IGNITE DMD trial, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 in patients with DMD, the Company’s regulatory plans, the Company’s SGT-003 program, including the Company’s expectation for filing an IND, timelines, the sufficiency of the Company’s cash and cash equivalents to fund its operations, and other statements containing the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” “working” and similar expressions. 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 the Company’s ability to continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; obtain and maintain the necessary approvals from investigational review boards at IGNITE DMD clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll additional patients in IGNITE DMD and on the timeline expected; the Company’s dosing strategy; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; whether the interim data referenced in this release will be predicative of the final results of the trial or will demonstrate a safe or effective treatment benefit of SGT-001; whether the methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters will yield meaningful statistical results; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully optimize and scale its manufacturing process; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, SGT-003 and other product candidates, achieve its other business objectives and continue as a going concern. 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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