Solid Biosciences Reports 1.5-Year Data from Patients in the Ongoing IGNITE DMD Phase I/II Clinical Trial of SGT-001

September 23, 2021

- Data support continued functional benefit 1.5 years post treatment compared with natural history data; assessed by North Star Ambulatory Assessment (NSAA), 6-Minute Walk Test (6MWT) and Forced Vital Capacity (FVC) -

- Patient reported outcomes showed sustained improvements at 1.5 years compared to patient baseline and natural history data -

- No new drug-related safety findings for a period of up to 3.5 years -

CAMBRIDGE, Mass., September 27, 2021 – Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), will report positive 1.5-year functional data and patient-reported outcome measures (Pediatric Outcomes Data Collection Instrument, or PODCI) for Patients 4-6 in the ongoing IGNITE DMD Phase I/II clinical trial of SGT-001. Vamshi Rao, MD, Attending Physician, Neurology, Lurie Children's Hospital of Chicago and Assistant Professor of Pediatrics (Neurology and Epilepsy), Northwestern University Feinberg School of Medicine, and an IGNITE DMD clinical investigator, will present the data today in an oral session at the World Muscle Society 2021 Virtual Congress.

"The data presented today demonstrate durable expression and function of microdystrophin protein in biopsy samples collected 12 to 24 months post-dosing of SGT-001," said Dr. Rao. "Additionally, these data provide encouraging evidence of functional benefit at 1.5 years post-treatment compared with natural history data and show meaningful improvement in patient-reported outcomes. Data from additional patients should enhance our understanding of the role that SGT-001 may play in improving outcomes for patients with Duchenne."

"We continue to believe that SGT-001 has the potential to provide differentiated benefit to patients with Duchenne and are on track to dose additional patients in IGNITE DMD," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences.

IGNITE DMD 1.5-Year Data

Today, the company will report 1.5-year functional data and patient-reported outcome measures for Patients 4-6, all of whom received 2E14 vg/kg of SGT-001 manufactured using Solid Biosciences' first-generation manufacturing process. The company previously reported 1-year data for the same measures in March 2021.

Biopsy Data

As previously reported in May 2021, analyses of the long-term biopsy data collected from Patients 4-6 at the 2E14 vg/kg dose level, taken 2 years, 1.5 years and 1-year post-dosing, respectively, indicate evidence of durable and widespread expression of the microdystrophin protein.

Functional Data

- NSAA scores at 1.5 years showed minimal change compared with baseline and suggest benefit after treatment when compared to trajectories typically observed in natural history data. Natural history analyses suggest that patients similarly aged to those enrolled in IGNITE DMD would normally be expected to exhibit a 4.5-point decline in NSAA over 1.5 years. In contrast, Patients 4-6 exhibited a mean decrease of 1.7 points (Range: -3 to 0 points) from baseline and a mean difference of +8 points compared with natural history data over the same time period.
- 6MWT distances were maintained 1.5 years post dosing, while natural history analyses suggest that similarly aged patients would normally be expected to exhibit a 63.5-meter decline over the same period. The mean increase in the 6MWT for Patients 4-6 at 1.5 years was 15.3 meters (Range: -17 to +56 meters) compared with baseline, and the mean difference compared with natural history data was +78.8 meters over the same period.
- The percent predicted FVC for Patients 4-6 continued to show stability or improvement 1.5 years following SGT-001 administration, while natural history analyses suggest that similarly aged patients would normally be expected to exhibit a decline of 7.5% over the same period. 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.

Patient-Reported Outcome Measures

Patient-reported outcome measures showed meaningful sustained improvements at 1.5 years compared with baseline as assessed using the PODCI global (range of change from baseline of +7 to +18 points), sports (Range: +14 to +23 points), transfer (Range: -6 to +3 points) and upper extremity scales (Range: +2 to +9 points). Data from natural history studies demonstrate a decline in the global (7.6 points), sports (4.7 points) and transfer (14.9 points) scales over the same period of time.

Safety Findings

No new drug-related safety findings have been reported in Patients 4-6, who have post-dosing periods of more than 1.5 years to 2.5 years, or any of Patients 1-8, who have post-dosing periods of more than 5 months to 3.5 years.

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.

Note: A previous version of this press release included inaccurate Forced Vital Capacity (FVC) data for Patient 5. The correction of that data impacted the FVC% range and mean. This version reflects the corrected data.

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