



Solid Biosciences Reports Efficacy and Safety Data from the Ongoing IGNITE DMD Clinical Trial and Resumption of Patient Dosing in the 2E14 vg/kg Cohort

March 15, 2021

- *Interim data from six patients provide evidence of a potential benefit of SGT-001 in functional endpoints of North Star Ambulatory Assessments (NSAA), 6-minute walk test (6MWT), pulmonary function tests (PFTs), and clinically validated patient reported outcome measures (PROMs) -*
- *Patient 7, safely dosed with SGT-001, experienced transient and manageable adverse events, none of which were serious; six patients previously dosed showed no new drug-related safety findings 17-37 months post dosing; screening and enrollment of patients into IGNITE DMD continue -*
- *Presentations to follow at the 2021 **Muscular Dystrophy Association (MDA)** Virtual Clinical & Scientific Conference -*
- *Company to host conference call and webcast **today at 4:30 PM ET** to discuss clinical data and financial results -*

CAMBRIDGE, Mass., March 15, 2021 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today reported encouraging interim functional (NSAA, 6MWT and PFTs) and biomarker data, and patient reported outcome measures (PROMs) from six patients after treatment in the ongoing IGNITE DMD Phase I/II clinical trial of its lead gene therapy candidate, SGT-001. The Company also announced that patient 7 in IGNITE DMD was safely dosed, with transient and manageable adverse events, none of which were serious. Patient 7 was the first patient dosed in IGNITE DMD under a previously reported clinical protocol amendment and using SGT-001 manufactured with its second-generation process. Additionally, the six patients previously dosed showed no new drug-related safety findings, 17-37 months post dosing. The totality of data collected, and the re-initiation of dosing, support the continued enrollment of patients into the IGNITE DMD study.

These data will also be presented in an oral session and at a company-sponsored symposium at the 2021 MDA Virtual Clinical & Scientific Conference on Thursday, March 18.

"The totality of the functional and biomarker data, as well as the patient reported outcome measures reported today suggest that SGT-001 may provide benefit to patients with Duchenne, a serious disease for which there is no cure," said Barry Byrne, M.D., Ph.D., Associate Chair of Pediatrics and Director of the Powell Gene Therapy Center at the University of Florida, and Principal Investigator of the IGNITE DMD clinical study. "I am particularly encouraged by these early data when compared with the natural history of this disease. I look forward to the continued enrollment in IGNITE DMD and evaluating the data as the study progresses."

"We are encouraged with the successful resumption of dosing in the IGNITE DMD trial under our amended clinical protocol and using SGT-001 manufactured with a second-generation process. The safe dosing of the seventh patient gives us increased confidence in our dosing strategy as we move forward with clinical development in the IGNITE DMD clinical trial. We are grateful to this patient and his family, and to all those who choose to participate in clinical trials," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "We look forward to continuing to dose patients and reporting clinical outcomes from additional patients in the second half of 2021."

IGNITE DMD Data

The data reported were collected from the first six patients dosed in IGNITE DMD 12 to 24 months after treatment and include data from three patients dosed at the low dose (5E13 vg/kg) and three patients dosed at the high dose (2E14 vg/kg). Data from the delayed treatment cohort, analyzed as an untreated control cohort, were evaluated alongside representative natural history data. The six patients ranged in age from five to 14-years-old at baseline. These data have been previously shared with FDA, as well with members of the IGNITE DMD Data Safety Monitoring Board and clinical consultants.

Functional Data

- Among patients in the low and high dose cohorts, North Star Ambulatory Assessment (NSAA) scores at one year suggest benefit after treatment as 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 year-over-year disease progression ranging from a plateau in gains to a 3 to 3.7-point decline. Patients in the untreated control cohort exhibited a mean decline of 4.0 points from baseline to 1 year, while patients in the low-dose cohort exhibited a mean improvement of 1.0 point over the same period of time. Patients in the high-dose cohort exhibited a mean improvement of 0.3 points as compared to their baseline values.
- Mean increase in the 6-Minute Walk Test (6MWT) distance was above the generally accepted minimally clinically important difference (MCID) of 30 meters in both the low- and high-dose cohorts after treatment. While patients in the untreated control cohort exhibited a decline of 8.5 meters from baseline to one year, patients in the low-dose cohort exhibited a mean improvement of 37 meters and patients in the high-dose cohort exhibited an improvement of 49.7 meters over the same period.
- With respect to pulmonary function tests (PFTs), the majority of patients in both dose groups exhibited improved forced

vital capacity (% predicted FVC) at one year when declines in pulmonary function would otherwise be typically observed in patients with Duchenne. From baseline to one year, patients in the untreated control cohort exhibited a mean decline of 10.7% on an absolute basis, while patients in the low-dose and high-dose cohorts exhibited a mean improvement of 3.9% and 16.7%, respectively, over the same period.

Biomarker Data

- As previously reported, biopsies of skeletal muscle three months after a single infusion of SGT-001 at a dose of 2E14vg/kg demonstrated widespread distribution of microdystrophin-positive muscle fibers with co-localization of neuronal nitric oxide synthase (nNOS) and β -sarcoglycan in the muscles of these patients.
- Creatine kinase (CK) assessments of the six patients provide potential physiological evidence of a positive or stabilizing effect after one year of treatment with a single high-dose infusion of SGT-001. An average sustained CK decline of approximately 50% in patients in the high-dose cohort was observed. In the low-dose cohort, an average CK increase of approximately 166% was observed, and in the control group an average CK increase of approximately 17% was observed.

Patient Reported Outcome Measures (PROMs) Data

Patient reported outcome measures taken after one year of treatment revealed a trend towards dose-ordered improvements in motor function subscales and fatigability assessments, providing real-world evidence to support the clinical and biomarker findings of varying degrees of benefit to patients in the low- and high- dose cohorts.

- Meaningful improvements were demonstrated in the Pediatric Outcomes Data Collection Instrument (PODCI), a validated PROM that contains questions to assess how caregivers and children evaluate the child's ability to walk, stand, and perform activities of daily living, as well as recreational activities. Motor function scores reflect the gains seen in 6MWT and benefit of NSAA observed in all dosed patients.
- Semi-structured, qualitative interviews conducted by Modus Outcomes Ltd with patients and caregivers about the impact of Duchenne on functioning demonstrated overall improvement in functional activity and school-related impacts (e.g., lower limb mobility, keeping up with peers, climbing stairs, sports) in low- and high-dose cohorts, with subjective decreased fatigability in all patients of both treatment cohorts.

As previously reported, three of the first six patients dosed prior to the protocol amendments introduced in 2020 developed four serious adverse events (SAEs). All prior SAEs have fully resolved, and no new drug-related safety findings have been identified with post-dosing follow up of 17-37 months. Additionally, as reported today, with resumption of dosing in IGNITE DMD, patient 7 was dosed safely with mild to moderate adverse events, all of which have fully resolved. The resumption of dosing was under an amended clinical protocol and using SGT-001 manufactured with an improved process, both of which are designed to enhance patient safety.

Data Presentations at MDA

Dr. Byrne will present the IGNITE DMD efficacy and safety data during a virtual oral session at the MDA conference on Thursday, March 18, 2021 at 4:00 PM ET. Also, on March 18, the PROM data will be presented during a Company-sponsored lunch symposium at 12:00 p.m. ET. Registration for the MDA conference is required to attend the oral presentation and the lunch symposium. Registration information is available at: <https://mdavirtualconference.org/en/registration>.

MDA Presentation Details

Presentation Title: IGNITE-DMD: Phase I/II Ascending Dose Study of Single IV Infusion of SGT-001 Microdystrophin Gene Therapy for DMD: One Year Efficacy and Safety Results

Presentation Date: Thursday, March 18, 2021, 4:00 PM ET

Symposium Details

Also, on March 18, Solid Biosciences will sponsor a symposium, "Real World Outcome Measures in Duchenne Muscular Dystrophy: Current and Novel Assessments of Meaningful Patient Benefit" at 12 PM ET. The symposium will feature:

- Valeria Ricotti, MD, Co-Founder, Executive Vice-President & Chief Medical Officer at DiNAQOR, Honorary Clinical Lecturer, Biomedical Research Centre, UCL GOS Institute of Child Health
- Chad R. Heatwole, MD, MS-CI, Professor of Neurology, Associate Director of the Center for Health + Technology (CHeT) and CHeT Outcomes Division Director at the University of Rochester Medical Center
- Craig M. McDonald, MD, Professor and Chair of the Department of Physical Medicine and Rehabilitation at the University of California, Davis

Conference Call Today at 4:30 PM ET

Management will host a webcast and conference call to review the IGNITE DMD data and will take questions on this data and Solid's financial results, today at 4:30 PM ET.

A live webcast of the call will be available on the Company's website at www.solidbio.com under the "News & Events" tab in the Investor Relations section, or by clicking [here](#). Participants may also access the call, by dialing 866-763-0341 for domestic callers or 703-871-3818 for international callers, referencing conference ID# 1669808.

The archived webcast will be available for in the "News and Events" section of the Company's website.

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 expectations for reporting future data from the IGNITE DMD trial, the Company's regulatory plans and timelines 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 or 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 patients in IGNITE DMD 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 presented 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, 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.

Investor Contact:

David Carey
FINN Partners
212-867-1768
David.Carey@finnpartners.com

Media Contact:

Erich Sandoval
FINN Partners
917-497-2867
Erich.Sandoval@finnpartners.com



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