Solid Biosciences Provides First Quarter 2021 Business Update and Financial Results

May 14, 2021

- Patients 7 and 8 dosed in IGNITE-DMD Phase I/II clinical trial under new clinical protocol and second-generation SGT-001 manufacturing process; patient 8 experienced a serious adverse event -

- Long-term biopsy data generated from the ongoing IGNITE DMD trial demonstrate durable microdystrophin expression as measured up to two years post dosing of SGT-001. Data to be presented today at ASGCT at 1:45 PM ET -

- Continued research and innovation in musculoskeletal therapy generates next-generation DMD gene transfer candidate, SGT-003 -

- Company ends Q1 with $268.5 million in cash; cash runway into Q4 2022 -

- Company to hold conference call at 8:30am ET today -

CAMBRIDGE, Mass., May 14, 2021 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today provided a first quarter 2021 business update, which includes updates on two additional patients dosed in the IGNITE DMD clinical trial, long term biopsy data from prior patients dosed in the high dose 2E14 vg/kg cohort of the trial, advancement of a next-generation gene therapy program for Duchenne and financial results for the quarter ended March 31, 2021.

"We are pleased to have progressed with dosing patients in our IGNITE DMD trial and will continue monitoring patient outcomes as discussions with our Data Safety Monitoring Board (DSMB) on the safety and efficacy profile of SGT-001 are ongoing. We also announced data that will be presented later today at the American Society of Gene & Cell Therapy (ASGCT) annual meeting that demonstrate sustained microdystrophin expression for up to two years post-dosing," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "We are excited by the durability of the microdystrophin protein observed in these patients, and the potential for the meaningful impact this may have on their lives."

IGNITE DMD Clinical Trial Update

Solid Biosciences has continued patient dosing in IGNITE DMD with SGT-001 using Solid's improved manufacturing process and under an amended clinical protocol designed to enhance patient safety. As previously reported. Patient 7 was dosed uneventfully and continues to do well. In April 2021, an eighth patient, also in the 2E14 vg/kg cohort, was treated with SGT-001. The patient experienced an inflammatory response which was classified as a serious adverse event and considered by the investigator to be drug-related. This type of event is described in our investigators brochure and is not considered unexpected. As of the patient's 30-day follow-up visit, laboratory values had either returned to normal or continue to trend towards normal.

As part of Solid's clinical mitigation strategy, the status of both patients dosed under the amended protocol have been shared with the Company's DSMB as well as the Food and Drug Administration (FDA). The Company is carefully examining the full details of the clinical course of these patients and is continuing its discussions with these two bodies. No new drug-related safety findings have been identified in Patients 1 through 6 who have post dosing periods of 1.5 years to more than 3 years.

IGNITE DMD Long Term Biopsy Data Update

Today the Company will share long-term biopsy data collected from patients 4-6 at the 2E14 vg/kg dose level. Analyses of the biopsies, taken 2 years, 1.5 years and 1-year post-dosing, respectively, indicate evidence of durable and widespread expression of the microdystrophin protein. The long-term results are consistent with the Day 90 data previously reported and continue to demonstrate the functionality of the SGT-001 microdystrophin, as highlighted by the recruitment of key dystrophin associated proteins: beta-sarcoglycan and neuronal Nitric Oxide Synthase (nNOS). The long-term muscle biopsy results were analyzed by two methods, Western Blot and Immunofluorescence (IF), and are provided in the table below:

Patient	Last Timepoint	Western Blot		Immunofluorescence (% Positive Fibers)	
		Day 90	Last Timepoint	Day 90	Last Timepoint
Pt. 4	24 months	BLQ*	BLQ	10-20%	10-30%
Pt. 5	18 months	17.5%	69.8%	50-70%	85%
Pt. 6	12 months	8.0%	20.3%	50-70%	50-60%

**Below the limit of quantification*

In summary, the Western Blot data indicate that expression was maintained in Patient 4 and increased compared to the Day 90 biopsies in Patient 5 and 6. IF data show microdystrophin function via continued localization to the muscle membrane, with the percent positive fibers remaining comparable to the Day 90 biopsies in all three patients.

Further morphological analyses of the muscle biopsies indicate that sustained microdystrophin protein expression and function resulted in membrane stabilization, evidenced by minimal progression of muscle deterioration since the Day 90 timepoint. These long-term pathophysiological improvements are potentially supportive of the recently reported positive trends in the clinical biomarker and functional data from the IGNITE DMD trial.

These data will be presented today by Dr. Carl Morris, PhD at 1:45 PM ET at the ASGCT 24th Annual Meeting (Abstract #263).

R&D Pipeline Update

Today Solid announced the advancement of a next-generation DMD microdystrophin gene transfer program, SGT-003. This program is an internally developed preclinical candidate leveraging Solid's broad expertise in gene therapy and muscle biology. Data presented at the ASGCT meeting (Abstract #319) demonstrate that Solid Biosciences has successfully developed a library of novel capsids that show increased muscle tropism, decreased liver biodistribution, and drive improved efficiency compared with AAV9 in various *in vitro* and *in vivo* models. SGT-003 is a preclinical candidate that combines a novel and rationally designed capsid candidate with Solid's proprietary nNOS-containing microdystrophin.

In addition to its internal research and development efforts, Solid also has an ongoing alliance with Ultragenyx to explore other next-generation opportunities to develop additional Duchenne gene therapies. The Companies have been collaborating to optimize candidate vectors that leverage the nNOS-containing microdystrophin construct with an AAV8-like capsid within the Ultragenyx HeLa producer cell line manufacturing approach.

Abstracts for the data being presented today at the ASGCT annual meeting can be viewed online at: https://annualmeeting.asgct.org/

Financial Highlights
Collaboration revenue for the first quarter of 2021 were $3.3 million, compared to no collaboration revenue for the three months ended March 31, 2020. The increase in collaboration revenue related to research services and cost reimbursement from the Collaboration Agreement with Ultragenyx.

Research and development expenses for the first quarter of 2021 were $14.2 million, compared to $19.7 million for the first quarter of 2020. The decrease was primarily attributable to a decrease in costs related to our lead product candidate SGT-001, driven by a reduction in manufacturing costs, clinical costs, and unallocated research and development costs.

General and administrative expenses for the first quarter of 2021 were $6.0 million, compared to $5.3 million for the first quarter of 2020. The increase was primarily attributable to increased personnel costs as the Company progressed it IGNITE DMD clinical trial.

Net loss for the first quarter of 2021 was $16.9 million, compared to $26.7 million for the first quarter of 2020.

Solid had $268.5 million in cash and cash equivalents as of March 31, 2021, which includes $143.8 million in aggregate gross proceeds, before deducting underwriting discounts and offering expenses, raised in an underwritten public offering in the first quarter of 2021. The Company expects that its cash and cash equivalents will enable Solid to fund its operating expenses and capital expenditures into the fourth quarter of 2022.

Conference Call Information
The company will host a conference call and webcast at 8:30 a.m. ET today to discuss the program update. Participants are invited to listen by dialing +1 866-763-0341 (domestic) or +1 703-871-3818 (international) five minutes prior to the start of the call and providing the passcode 4772539. A listen-only webcast of the conference call can also be accessed through the "Investors" tab on the Solid Biosciences website, www.solidbio.com, and a replay of the call will be available for approximately six weeks after the call.

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, the Company's SGT-003 program, 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, 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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