Solid Biosciences Provides Third Quarter 2021 Business Update and Financial Results

November 3, 2021

- Continued focus on advancing SGT-001 includes additional patient dosings in IGNITE DMD expected to begin in Q4 2021 -

- Continued progress with SGT-003; Solid's next-generation Duchenne gene therapy program demonstrates enhanced muscle tropism and microdystrophin expression -

- Company ends Q3 with approximately $229.8 million in cash and investments; cash runway into Q2 2023 -

CAMBRIDGE, Mass., Nov. 03, 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 business update as well as financial results for the third quarter ended September 30, 2021.

"Solid made progress across all its strategic priorities during the third quarter. We expect to dose additional patients in IGNITE DMD beginning in the fourth quarter of 2021. We also shared meaningful clinical data across a variety of endpoints from the ongoing IGNITE DMD clinical trial of SGT-001, which continue to guide our understanding of the potentially differentiated benefit that SGT-001 may offer to patients. In addition, we advanced our Duchenne pipeline program, SGT-003, including selecting a manufacturing approach that will allow us to quickly move into human proof of concept in early 2023," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "As we prepare to dose additional patients in IGNITE DMD, we enter the fourth quarter with a great deal of excitement and momentum around our opportunities to improve outcomes for patients with Duchenne."

SGT-001 Update
Solid expects to dose additional patients in IGNITE DMD, beginning in the fourth quarter of 2021. In addition, the company expects to report 90-day biopsy data from the first three patients dosed with SGT-001 manufactured with its improved process as well as long-term expression and functional data in early 2022. Concurrent with activities to support continued patient dosing in IGNITE DMD, Solid is also engaged in a variety of activities to support continued advancement of SGT-001, including scaling its improved manufacturing process.

In September 2021, the Company reported 1.5-year data from IGNITE DMD at the World Muscle Society 2021 Virtual Congress and additional pulmonary function results from IGNITE DMD at the Child Neurology Society 50th Annual Meeting. Patients dosed with SGT-001 exhibited sustained benefit across all reported measures over a period when natural history would suggest a decline. Solid is taking a leading role in reporting long-term respiratory data showcasing stability or improvement in pulmonary function across forced vital capacity, peak expiratory flow and forced expiratory volume in one second, important measures in the progression of Duchenne in patients. These data continue to suggest SGT-001 is providing benefit to dosed patients.

R&D Pipeline Update
SGT-003, Solid's next-generation adeno-associated virus (AAV) capsid pipeline program, continued its progress during the third quarter and continues to demonstrate enhanced muscle tropism and microdystrophin expression in preclinical studies. Biodistribution data show increased vector genomes in muscle and heart as well as decreased vector genomes in liver with SGT-003 compared to AAV9 in various *in vitro* and *in vivo* models. This biodistribution profile has the potential to increase efficiency and specifically target muscle cells, which could potentially allow for a reduced total viral load.

As announced last month, Solid has entered into an agreement with Forge Biologics to advance the development and manufacturing of SGT-003 using a transient transfection process. Solid is working closely with Forge to advance manufacturing of SGT-003 and is looking toward a target IND filing in early 2023.

Solid also continued its progress with Ultragenyx on the companies' collaboration that leverages Solid's proprietary nNOS-containing microdystrophin construct with an AAV8-like capsid within Ultragenyx's HeLa producer cell line manufacturing approach. A program update is expected before the end of 2021.

Recent Company Developments
Solid, in collaboration with REGENXBIO formally launched the Pathway Development Consortium (PDC), a multistakeholder initiative which aims to identify, develop, expand and maintain pathways to effective therapies for patients diagnosed early in life with rare diseases. The PDC seeks to achieve these goals by bringing together a broad and diverse group of stakeholders from the rare disease and AAV gene therapy communities, including patients, industry, regulators, academia and payers, among others, for meaningful scientific and policy discussions.

Financial Highlights
Collaboration revenue for the third quarter of 2021 was $3.5 million, compared to no collaboration revenue for the third quarter of 2020. The increase in collaboration revenue is related to research services and cost reimbursement from our Collaboration Agreement with Ultragenyx, which the Company entered into in the fourth quarter of 2020.

Research and development expenses for the third quarter of 2021 were $14.4 million, compared to $16.0 million for the third quarter of 2020. The decrease was primarily related to lower manufacturing expenses for our lead product, SGT-001, offset by an increase in clinical and other R&D costs.

General and administrative expenses for the third quarter of 2021 were $7.1 million, compared to $5.2 million for the third quarter of 2020. The increase was primarily attributable to increased corporate and personnel-related expenses.

Net loss for the third quarter of 2021 was $18.0 million, compared to $21.2 million for the third quarter of 2020.

Solid had approximately $229.8 million in cash, cash equivalents and available-for-sale securities as of September 30, 2021. As a result of the pace of patient dosing in IGNITE DMD and timing of anticipated manufacturing expenses, Solid now expects that its cash, cash equivalents and available-for-sale securities will enable the Company to invest in its Duchenne gene therapy programs and capital expenditures into the second quarter of 2023.

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 SGT-003
SGT-003, Solid's next-generation gene therapy candidate for the treatment of Duchenne, utilizes a rationally designed AAV-based vector to deliver the proprietary and differentiated microdystrophin construct that is also incorporated into SGT-001. SGT-003 has demonstrated improved biodistribution compared with AAV9 in various *in vitro* and *in vivo* models, with increased delivery to and expression in skeletal and heart muscle and reduced tropism for liver cells. Solid is targeting an IND filing in early 2023.

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, as well as our recently announced next-generation gene therapy candidate, SGT-003. 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 Company's plans to represent data from IGNITE DMD, 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, cash equivalents and available-for-sale securities 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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