Solid Biosciences Provides Second Quarter 2021 Business Update and Financial Results

August 16, 2021

- Clinical activities underway to advance patient dosing in IGNITE DMD; next patient dosing anticipated in Q4 2021 -

- Development activities continue for pipeline initiatives SGT-003 and Ultragenyx collaboration, next generation gene therapy programs for Duchenne patients -

- Company ends Q2 with approximately $249 million cash and investments; cash runway into Q4 2022 -

CAMBRIDGE, Mass., Aug. 16, 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 second quarter 2021 business update as well as financial results for the quarter ended June 30, 2021.

"We continue on our mission of developing meaningful treatments for patients with Duchenne," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "During the second quarter, we made progress on our key strategic priorities and strengthened our team. Specifically, we advanced clinical activities to prepare for the next patient to be dosed in the IGNITE DMD clinical trial of SGT-001, furthered our next generation Duchenne gene therapy program, SGT-003, and progressed our Duchenne collaboration with Ultragenyx. We also continue to maintain a strong balance sheet to support further investment into our portfolio of Duchenne programs."

IGNITE DMD Clinical Trial Update
As previously reported, in April 2021, the eighth patient in IGNITE DMD, and fifth patient in the 2E14 vg/kg cohort, was treated with SGT-001. The patient experienced an SAE which has since fully resolved.

Following dosing of two patients with Solid's second-generation manufacturing process and clinical strategy, Solid conducted an extensive review of all clinical data, resulting in a strengthened risk mitigation plan including new patient management guidance, which has also been submitted to the FDA. Activities are underway to advance IGNITE DMD with the next patient dosing anticipated in the fourth quarter of 2021.

No new drug-related safety findings have been identified in Patients 1 through 8 in post-dosing periods of 90-days to more than 3 years. The Company continues to follow dosed patients and collect data to support the potential benefit from dosing SGT-001.

R&D Pipeline Update
Preclinical activities progressed on Solid's internally developed next generation Duchenne microdystrophin gene transfer program, SGT-003, including proof of concept research; manufacturing, regulatory and clinical strategies; and development and validation of screening assays to support clinical dosing. The Company is targeting an IND submission for SGT-003 in early-2023.

Solid also continued to work in partnership with Ultragenyx during the quarter to further the companies' ongoing collaboration, which is focused on optimizing candidate vectors that leverage Solid's proprietary nNOS-containing microdystrophin construct with an AAV8-like capsid within Ultragenyx's HeLa producer cell line manufacturing approach.

Recent Company Developments

- Solid strengthened its leadership team with recent additions in the areas of Regulatory Sciences, Clinical Development, Communications & Investor Relations and Legal & Intellectual Property.
- Solid established a Technical Advisory Board comprised of leaders with track records of success in biopharmaceutical product development. The advisory board members will provide strategic input on technical and CMC aspects of advancing SGT-001 and SGT-003 through clinical development and toward commercialization.

Financial Highlights
Collaboration revenue for the second quarter of 2021 was $3.6 million, compared to no collaboration revenue for the three months ended June 30, 2020. The increase in collaboration revenue is related to research services and cost reimbursement from our Collaboration Agreement with Ultragenyx, which we entered into in the fourth quarter of 2020.

Research and development expenses for the second quarter of 2021 were $15.5 million, compared to $13.4 million for the second quarter of 2020. The increase was primarily attributable to increased costs related to our lead product candidate, SGT-001, driven by an increase in manufacturing costs, clinical costs and other research and development costs related to other product candidates.

General and administrative expenses for the second quarter of 2021 were $6.8 million, compared to $5.5 million for the second quarter of 2020. The increase was primarily attributable to increased corporate and personnel-related expenses.

Net loss for the second quarter of 2021 was $18.7 million, compared to $19.0 million for the second quarter of 2020.

Solid had approximately $249.0 million in cash, cash equivalents and available-for-sale securities as of June 30, 2021. The Company expects that its cash, cash equivalents and available-for-sale securities will enable Solid to invest in its Duchenne gene therapy programs and capital expenditures into the fourth quarter of 2022.

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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