Solid Biosciences Provides Second Quarter 2022 Business Update and Financial Results

August 11, 2022

- Transition to transient transfection-based manufacturing process continues for SGT-001; Company expects to continue dosing patients in 2023 -

- Development of next-generation Duchenne gene therapy candidate SGT-003 continues; Company anticipates mid-2023 Investigational New Drug (IND) submission -

- Company ends Q2 with approximately $162.9 million in cash and investments; cash runway into Q2 2024 -

CHARLESTOWN, Mass., Aug. 11, 2022 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today reported financial results for the second quarter ended June 30, 2022 and provided a business update.

“Solid has made important progress on both of our gene therapy programs this year,” said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. “We remain focused on executing on our development plans and hitting key milestones. We anticipate sharing long-term data from the Phase 1/2 IGNITE DMD trial of SGT-001 in early 2023. We expect that these data will further inform our understanding of how patients have benefitted from SGT-001 over time and are optimistic that longer-term results will reinforce the durability of response that we have seen in patients who were treated with SGT-001.”

SGT-001 Update
In April 2022, Solid announced that, following a robust manufacturing analysis, the company would streamline operations and make a strategic shift to a commercially scaled transient transfection-based manufacturing process. Development activities, including manufacturing scale-up, additional nonclinical testing and regulatory discussions with the Food and Drug Administration (FDA) are ongoing. The company expects to continue dosing patients using SGT-001 in 2023 with product made using the new transient-based process. The company also expects to share additional data from IGNITE DMD in early 2023, including the study’s primary one-year analysis of all treated patients as well as three-year longitudinal data from Patients Four through Six.

SGT-003 Update
Development activities continued in the quarter for Solid’s next generation Duchenne gene therapy candidate, SGT-003, including manufacturing scale-up, IND enabling preclinical studies and engagement with the FDA. This candidate combines Solid’s differentiated nNOS microdystrophin with the capsid AAV-SLB101, a novel, rationally designed AAV that was screened in Solid’s internal development platform of capsids designed to improve transduction to muscle tissue. As reported in April 2022, data from a non-human primate study using a reporter transgene in AAV-SLB101 demonstrated increased muscle tropism, decreased liver biodistribution and improved efficiency compared with AAV9. The company is on track for an anticipated mid-2023 Investigational New Drug (IND) submission for SGT-003.

Second Quarter 2022 Financial Highlights
Collaboration revenue for the second quarter of 2022 was $6.2 million, compared to $3.6 million for the second quarter of 2021. Collaboration revenue in both periods was 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 second quarter of 2022 were $23.2 million, compared to $15.5 million for the second quarter of 2021.

General and administrative expenses for the second quarter of 2022 were $6.9 million, compared to $6.8 million for the second quarter of 2021.

Net loss for the second quarter of 2022 was $25.1 million, compared to $18.7 million for the second quarter of 2021.

Solid had $162.9 million in cash, cash equivalents and available-for-sale securities as of June 30, 2022, compared to $207.8 million as of December 31, 2021. The company expects that its cash, cash equivalents and available-for-sale securities will enable Solid to fund its operations and capital expenditures into the second quarter of 2024.

About SGT-001
Solid’s SGT-001 is an 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. Solid’s proprietary and differentiated nNOS 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 is Solid's next-generation AAV gene transfer therapy candidate that utilizes a rationally designed, novel muscle-tropic AAV capsid, called AAV-SLB101, to deliver Solid’s proprietary and differentiated nNOS microdystrophin for the treatment of Duchenne. AAV-SLB101 has demonstrated enhanced muscle biodistribution and transgene expression, as well as reduced liver tropism, compared with AAV9 in *in vivo* mouse models and, utilizing a reporter transgene, non-human primate *in vivo* models. SGT-003 has correspondingly demonstrated higher levels of microdystrophin expression in vivo in the mdx mouse model of Duchenne and in vitro in human Duchenne cell lines. Solid is targeting an Investigational New Drug submission for SGT-003 in mid-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 gene therapy candidates, SGT-001 and 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 Company's plans to present data from IGNITE DMD, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 or SGT-003 in patients with Duchenne, the Company's regulatory plans and discussions, the Company's plan to continue dosing with SGT-001, 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 advance its SGT-001 and SGT-003 programs on the timelines 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 clinical trial sites and independent data safety monitoring board; replicate in clinical trials positive results found in preclinical studies and early-stage clinical trials of its product candidates; 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 or SGT-003; whether the methodologies, assumptions and applications the Company utilizes 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 transition, 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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