



Solid Biosciences Announces Updated Corporate Strategy to Develop SGT-001 and SGT-003 Pipeline Programs for Patients with Duchenne Muscular Dystrophy

April 27, 2022

- IGNITE DMD functional and durability patient data support advancement of SGT-001; Program transitioning to a commercially scaled transient transfection-based manufacturing process -
- Novel capsid development to continue, supported by new preclinical data; Company anticipates early-2023 Investigational New Drug (IND) submission for SGT-003 for Duchenne -
- Strategy and resource alignment support funding of operations through important clinical milestones and into Q2 2024 -
- Solid Biosciences reports first quarter 2022 financial results; Company to hold conference call at 8:00am ET today -

CAMBRIDGE, Mass., April 27, 2022 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today announced an update to its strategic priorities to focus on developing SGT-001 and SGT-003. The company also announced financial results for the first quarter ended March 31, 2022.

"Today, we are sharing changes to our corporate strategy to better align our resources behind Solid's core values of innovation and patient centricity, with a focus on bringing our differentiated microdystrophin gene therapy to more patients. We will be transitioning SGT-001 to a commercially scaled manufacturing process, advancing SGT-003 toward an anticipated IND submission in early 2023, and aligning our organization behind these two programs," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences.

Mr. Ganot continued, "Recent data suggest that our lead program, SGT-001, holds meaningful promise as a potential treatment option. In addition, today we are sharing new preclinical data which suggest that Solid's novel capsid may offer enhanced muscle tropism compared with AAV9, and has the potential to benefit patients with Duchenne as well as the broader gene therapy landscape for muscle-related disorders."

SGT-001 Update

On March 14, 2022, [Solid released data](#) demonstrating that patients showed sustained benefit compared with natural history trajectories two years after treatment with SGT-001 across functional, pulmonary and patient reported outcome measures.

Manufacturing Update

As the company focuses on advancing SGT-001, it will be streamlining operations and making the strategic shift to a commercially scaled transient transfection-based manufacturing process. Following a robust manufacturing analysis, Solid believes that a new, outsourced process may provide improvements to manufacturability as well as additional organizational efficiencies.

Clinical Update

The company also announced that it has concluded enrollment in IGNITE DMD, its Phase 1/2 clinical trial for SGT-001, and will continue monitoring dosed patients for five years post-treatment. The company anticipates that future patients will be treated with SGT-001 manufactured using the new transient transfection-based process. Solid currently expects to continue dosing with SGT-001 in 2023.

SGT-003 Update

Today, Solid released new preclinical data suggesting that a novel, next generation capsid candidate may have meaningful advantages for the delivery of muscle-related gene therapies. New data from a non-human primate study using a reporter transgene in Solid's novel capsid demonstrated increased muscle tropism, decreased liver biodistribution and improved efficiency compared with AAV9. These results are consistent with earlier *in vitro* and *in vivo* studies in both dystrophic (MDX) and wild type mouse models, which suggested improved muscle tropism with Solid's novel capsid as well as improved expression of Solid's microdystrophin compared with AAV9. Solid's novel, muscle tropic capsid has been combined with the company's differentiated microdystrophin for the SGT-003 program for Duchenne. The company remains on track for an early 2023 IND submission for SGT-003.

Strategic Prioritization, Corporate Update and Financial Position

The company also announced a reorganization of its corporate operations to prioritize the advancement of its key programs, SGT-001 and SGT-003. Solid anticipates that the use of transfection-based manufacturing processes for both SGT-001 and SGT-003 will allow the company to focus its operating structure and better leverage external manufacturing expertise. In addition, the company plans to narrow Research & Development activities to those related to SGT-001, SGT-003 and next generation capsids. In connection with these activities, the company will reduce its headcount by approximately 35 percent. The reorganization will result in a reduction in planned corporate expenditures which is anticipated to extend funding of operations through important clinical milestones and into Q2 2024.

Joel Schneider, Ph.D., Solid's Chief Operating Officer, will also be leaving the company at the end of May to accept a role as the Chief Executive Officer for a privately held novel, viral-based gene therapy platform company.

"The strategic decision to streamline our manufacturing operations will allow us to consolidate our resources to support both SGT-001 and SGT-003 to important clinical milestones. I would like to extend my thanks and appreciation to the team members who were impacted by this reorganization. Each individual has made important contributions toward our mission of improving the lives of patients with Duchenne," said Mr. Ganot.

Mr. Ganot added, "I would also like to thank Joel for his leadership and service to Solid over the last eight years, and for the instrumental role he played to advance the field of gene therapy for Duchenne towards scientific translation and clinical development, as well as the possibilities it brings to patients worldwide."

First Quarter 2022 Financial Highlights

Collaboration revenue for the first quarter of 2022 was \$1.9 million, compared to \$3.3 million for the first 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 first quarter of 2022 were \$19.9 million, compared to \$14.2 million for the first quarter of 2021.

General and administrative expenses for the first quarter of 2022 were \$7.4 million, compared to \$6.0 million for the first quarter of 2021.

Net loss for the first quarter of 2022 was \$25.3 million, compared to \$16.9 million for the first quarter of 2021.

Solid had \$180.1 million in cash, cash equivalents and available-for-sale securities as of March 31, 2022. 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.

Conference Call Information

The company will host a conference call and webcast at 8:00 a.m. ET today to discuss the strategic 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 Conference ID or Passcode 6545417. A live 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 SGT-003

SGT-003, Solid's next-generation gene therapy candidate for the treatment of Duchenne, utilizes a rationally designed, novel capsid candidate 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 Investigational New Drug submission 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 reduction in headcount, reduction in corporate expenses 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, 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 successfully implement its headcount reduction and reduce expenses; the impact of the headcount reduction on the Company's business; 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 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.

Investor Contact:

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

Caitlin Lowie
Solid Biosciences
607-423-3219
clowie@solidbio.com

Media Contact:

Erich Sandoval

FINN Partners

917-497-2867

Erich.Sandoval@finnpartners.com



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