



Solid Biosciences Outlines its Strategic Priorities for 2022 and Announces Corporate Updates

January 10, 2022

- Continue to advance SGT-001 by dosing additional patients in IGNITE DMD -
- Advance next-generation Duchenne gene therapy program (SGT-003) to IND submission; SGT-003 has demonstrated enhanced muscle tropism and microdystrophin expression -
- Company enters 2022 with approximately \$210 million in cash and investments -
- Ian F. Smith, Chair of the Board of Directors, to assume role of Executive Chair -

CAMBRIDGE, Mass., Jan. 10, 2022 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today provided an update on its 2022 strategic priorities and other business initiatives in advance of its presentation at the 40th Annual J.P. Morgan Healthcare Conference scheduled for Thursday, January 13 at 9am ET.

"2022 will be an important year for Solid as we look to advance genetic medicines for Duchenne. We intend to dose additional patients in the IGNITE DMD Phase I/II clinical trial of SGT-001 utilizing our updated risk mitigation strategy and second-generation manufacturing process. In the first half of 2022 we expect to share additional data from IGNITE DMD, including data from Patient 9, who was dosed in November 2021. We are also moving our next generation Duchenne program, SGT-003, to an IND submission anticipated in early 2023. Based on the preclinical work completed to date demonstrating enhanced muscle tropism and microdystrophin expression, we believe SGT-003 has the potential to offer further benefit to patients with Duchenne," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "We enter 2022 with approximately \$210 million in cash and investments, which we expect will support continued progress of the SGT-001 clinical development activities to enable the completion of IGNITE DMD and discussions with regulatory bodies, as well as advancement of SGT-003 to the clinic."

Highlights from the presentation to be given at the J.P. Morgan Healthcare Conference include:

- **SGT-001:** Following implementation of the updated risk mitigation strategy, the Company dosed a 9th patient in the IGNITE DMD Phase I/II clinical trial for SGT-001 in November 2021 using the company's second-generation manufacturing process. Solid plans to continue dosing patients in IGNITE DMD in 2022 as well as share additional expression, functional, pulmonary and patient reported outcomes data from the trial in the first half of this year.
- **SGT-003:** Solid will present additional data on SGT-003 demonstrating increased protein expression and more targeted biodistribution compared to AAV9. The company also intends to initiate IND-enabling studies in 2022 to support an IND submission in early 2023.
- **Platform Technologies:** The company will introduce and present data on development programs for two Platform Technologies, Novel Capsids and Dual Gene Expression (DGE). These programs are part of the company's ongoing research efforts to develop innovative technologies that Solid believes may hold potential to translate into meaningful treatments and drive the company's future pipeline expansion.

The company is also announcing that the Chair of its Board of Directors, Ian F. Smith, has been named Executive Chair. Mr. Smith is a highly accomplished life sciences executive with more than 20 years of finance and operating leadership experience with public and private biopharmaceutical companies. The appointment as Executive Chair reflects the relationship Mr. Smith has developed with Solid since joining the company's Board of Directors in April 2020. As Board Chair, Mr. Smith has worked in close partnership with Mr. Ganot and other members of the Solid Executive Leadership Team to advance Solid's near-term priorities. Formalizing the relationship as Executive Chair will allow Mr. Smith to provide additional leadership support as the company continues to develop novel technologies to support meaningful treatments for patients.

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.

Financial Information and Forward-Looking Statements

The preliminary financial information presented in this press release is unaudited and based on currently available information, may be adjusted as a result of the completion of customary quarterly and annual review and audit procedures, does not present all necessary information for a complete understanding of the company's financial condition as of December 31, 2021 or the company's results of operations for the year ended December 31, 2021, and the company's actual financial results may differ materially from the preliminary estimated financial information set forth above.

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 present 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 predictive 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 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 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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