Solid Biosciences Outlines Strategy for Leadership in Precision Genetic Medicines with Focus on Neuromuscular and Cardiac Diseases

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– Investigational New Drug (IND) application for SGT-003, next-generation gene therapy for Duchenne muscular dystrophy utilizing novel capsid AAV-SLB101, expected in 2H-2023 –

– Candidate selection and transition manufacturing process to transient transfection for AVB-202 for Friedrich's Ataxia and AVB 401 in dilated cardiomyopathy continue –

– Kevin Tan named Chief Financial Officer, experience to support execution of corporate strategy –

– Company enters 2023 with approximately $214 million in cash and investments; expected to fund key strategic priorities into 2025 –

CHARLESTOWN, Mass., Jan. 10, 2023 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company developing genetic medicines for neuromuscular and cardiac diseases, today outlined its strategic priorities for 2023. These announcements are being made in advance of Solid's presentation at the 41 st Annual J.P. Morgan Healthcare Conference scheduled for January 12 at 9:00 am PT.

"Solid has the people, pipeline, and processes to be a leader in precision genetic medicines for neuromuscular and cardiac diseases, and multiple anticipated key milestones across our expanded pipeline in 2023 and 2024 that will demonstrate our ability to execute on our strategy to build a platform-based company that may create meaningful genetic medicines in the company's future," said Bo Cumbo, President and Chief Executive Officer of Solid Biosciences. "The Solid leadership team has deep expertise in precision genetic medicine, and our diversified pipeline targets diseases with high unmet need, strong scientific rationale, and significant commercial potential. We are also developing novel capsid libraries that have applicability across our current disease focus, and we have a robust, scalable transient transfection-based platform, built on our experiences of manufacturing a number of different gene therapy programs; these can also be leveraged for current and future programs. Solid also enters 2023 with approximately $214 million in cash and investments, which is expected to support these priorities into 2025."

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

- Solid's corporate vision to build an innovation platform enabling the discovery and development of high-value genetic medicines for neuromuscular and cardiac diseases by integrating internal capabilities, including a vector core, validated animal models, optimized expression cassettes, novel capsids and regulatory elements of target indications, and collaborations with leaders in related clinical and research fields.

- Solid remains focused on developing next-generation AAV capsid libraries with two strategies designed to enhance cardiac and skeletal muscle tropism.
    - AAV-SLB101, the lead candidate from Solid's rationally designed novel capsid program and the vector used in the company's next-generation genetic medicine for Duchenne muscular dystrophy (SGT-003), has demonstrated more than double the transduction and expression in skeletal and cardiac muscle and half reduction in expression in the liver compared with AAV9 in non-human primate (NHP) studies, using a constitutive promoter and reporter gene.
    - SGT-003 has been well tolerated across multiple animal models and experience to date supports robust and scalable manufacturability currently up to 1000L.

- Friedrich's Ataxia (FA) program focused on drug candidate selection and initiation of IND enabling studies
    - FA, which results from loss of functional frataxin (FXN) protein and has neurological and cardiac effects, is a disease with significant unmet need and no approved therapies.
    - AVB-202 will be designed to express functional FXN protein
    - NHP studies conducted with AVB-202 manufactured using an HSV-based manufacturing method demonstrated that administration of AVB-202 led to FXN expression to significant levels in all tissues evaluated (heart, quadriceps, dorsal root ganglion, spinal cord, cerebellum, and deep cerebellar nucleus). In preclinical studies, AVB-202 resulted in safe levels of human FXN at or near physiologic levels in the NHP heart.

- BAG3-mediated Dilated Cardiomyopathy (DCM) AVB-401 program overview
    - Clear mechanistic rationale between genetic BAG3 insufficiency and myofilament damage, poor contraction and heart failure
    - Treating BAG3-mediated DCM requires expression of functional BAG3 within heart muscle only
    - Solid's construct candidates utilize the AAVrh74 capsid and cardiac specific promoter.
    - In early nonclinical studies, the specific capsid and cardiac-specific promoter combination increased cardiac

expression while reducing expression in the liver.
    - Solid is conducting additional studies to evaluate the potential of using AAV-SLB101 to develop a genetic medicine for BAG3-mediated DCM

- Strategic manufacturing overview
    - Solid's internal technical expertise and capabilities are complemented by a robust network of external partners who provide customized and dedicated resources to support advancement of the company's preclinical and early-stage pipeline programs.
    - Previously reported process change from HSV-based to transient transfection (TT)-based manufacturing has yielded a greater than two-fold increase in microdystrophin expression in mice for SGT-003 (TT) compared with SGT-001 (HSV).

- Upcoming anticipated milestones:
    - Report functional data from IGNITE DMD in early 2023
    - Submit SGT-003 IND in the second half of 2023
    - Initiate SGT-003 patient dosing in late 2023

As of December 31, 2022, Solid has approximately $214 million in cash and investments, which is expected to fund key strategic priorities into 2025.

The company also announced the appointment of Kevin Tan, CFA, as Chief Financial Officer.

About Solid Biosciences
Solid Biosciences is a life science company focused on advancing a portfolio of neuromuscular and cardiac programs, including SGT-003, a differentiated gene transfer candidate, for the treatment of Duchenne, AVB-202, a gene transfer candidate for the treatment of Friedreich's Ataxia, AVB-401 for BAG3-mediated dilated cardiomyopathy, and additional assets for the treatment of undisclosed cardiac diseases. Solid aims to be the center of excellence, bringing together those with expertise in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, Solid's mandate is to improve the daily lives of patients living with these devastating diseases. 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, 2022 or the company's results of operations for the year ended December 31, 2022, 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 future expectations, plans and prospects for the company; the anticipated milestones, business focus and pipeline of the company; the cash runway of the company and the sufficiency of the company's cash and investments to fund its operations; the company's SGT-003 program, including expectations for filing an IND and initiating dosing, AVB-202 program, including expectations for filing an IND, and AVB-401 program; the company's plans to present data from IGNITE DMD; the implication of preclinical data; 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 ability to recognize the anticipated benefits of Solid's acquisition of AavantiBio; the outcome of any legal proceedings that may be instituted against Solid or AavantiBio following the announcement of the acquisition and related transactions; the ability to obtain or maintain the listing of the common stock of the combined company on the Nasdaq Stock Market following the acquisition; the company's ability to advance its SGT-003, AVB-202, AVB-401 and other 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 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 and Friedreich's ataxia treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-003, AVB-202, AVB-401 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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