Solid Biosciences Granted FDA Orphan Drug Designation for Duchenne Muscular Dystrophy Gene Therapy Candidate SGT-003

January 16, 2024

– Patient dosing expected to begin mid-to-late Q1 2024 –

CHARLESTOWN, Mass., Jan. 16, 2024 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company developing precision genetic medicines for neuromuscular and cardiac diseases, today announced that it has been granted orphan drug designation (ODD) from the U.S. Food and Drug Administration (FDA) for SGT-003, the company's next-generation Duchenne muscular dystrophy (Duchenne) gene therapy candidate.

"Obtaining ODD status for SGT-003, along with Fast Track Designation granted last month, furthers our efforts to meet the ongoing challenge of treating this devastating disease as expeditiously as possible," said Bo Cumbo, President and Chief Executive Officer at Solid Biosciences. "These designations are important milestones for Solid, supporting the continued development of next-generation therapies for Duchenne."

The company is currently in the process of securing approvals from the institutional review boards (IRB) at the clinical trial sites for the planned Phase 1/2 clinical trial of SGT-003 and expects to commence patient screening shortly thereafter. Patient dosing in the trial is expected to commence in mid-to-late first quarter of 2024.

"SGT-003 therapy stands out among other Duchenne gene therapy candidates by leveraging a novel capsid and a muscle tropic vector delivering a microdystrophin that incorporates a neuronal Nitric Oxide Synthase (nNOS) binding domain. These attributes among others, have the potential to yield both more potent transduction than historical approaches, and a microdystrophin that may be able to more fully address muscle resiliency," said Gabriel Brooks, M.D., Chief Medical Officer at Solid Biosciences. "Obtaining ODD status is an exciting development that we believe will aid our efforts to bring advanced treatment options to those patients affected by Duchenne."

About SGT-003
SGT-003 uses a proprietary, rationally designed capsid (AAV-SLB101) to deliver a DNA sequence encoding a shortened form of the dystrophin protein (microdystrophin), containing the R16-R17 nNOS binding domain. Preclinical data suggests this may be important for both muscular function and durability of benefit in patients.

About Orphan Drug Designation
The FDA's Office of Orphan Products Development grants orphan designation status to drugs and biologics that are intended to treat a rare disease or condition that affects fewer than 200,000 people in the U.S. Orphan drug designation provides certain benefits, including specified financial incentives, to support clinical development and the potential for up to seven years of market exclusivity in the U.S. upon regulatory approval.

About DMD
Duchenne is a genetic muscle-wasting disease predominantly affecting boys, with symptoms usually appearing between three and five years of age. Duchenne is a progressive, irreversible, and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated prevalence of 5,000 to 15,000 cases in the United States alone.

About Solid Biosciences
Solid Biosciences is a life sciences company focused on advancing a portfolio of gene therapy candidates and neuromuscular and cardiac programs, including SGT-003, for the treatment of Duchenne muscular dystrophy (Duchenne), SGT-501 for the treatment of catecholaminergic polymorphic ventricular tachycardia (CPVT), AVB-401 for the treatment of BAG3-mediated dilated cardiomyopathy, AVB-202-TT for the treatment of Friedreich's ataxia, and additional assets for the treatment of fatal cardiac diseases. Solid is advancing its diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management, and care. Patient-focused and founded by those directly impacted, Solid's mandate is to improve the daily lives of patients living with these devastating diseases. 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 future expectations, plans and prospects for the company; the ability to successfully achieve and execute on the company's priorities and achieve key clinical milestones; the company's SGT-003 program, including securing IRB approval at clinical sites, initiating patient screening and initiating dosing, and the potential benefits of SGT-003; the potential benefits of ODD status; 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 company's ability to advance SGT-003, SGT-501, AVB-401, AVB-202-TT and other preclinical programs and capsid libraries on the timelines expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; replicate in clinical trials positive results found in preclinical studies of the company's product candidates; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne and other neuromuscular and cardiac treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-003, SGT-501, AVB-401, AVB-202-TT and other 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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Source: Solid Biosciences Inc.