Solid Biosciences Announces IND Clearance by FDA for Duchenne Muscular Dystrophy Gene Therapy Candidate SGT-003

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– Planning to initiate Phase 1/2 trial in pediatric DMD Patients –

– First cohort to study patients 4 to < 6 years of age –

CHARLESTOWN, Mass., Nov. 14, 2023 (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 received U.S. Food and Drug Administration (FDA) clearance of an Investigational New Drug (IND) application for SGT-003, the company's next-generation Duchenne Muscular Dystrophy (Duchenne) gene therapy candidate.

"We are pleased to have the FDA's clearance to proceed into the clinic with SGT-003, a new, innovative gene therapy candidate for Duchenne," said Bo Cumbo, President and CEO at Solid Biosciences. "SGT-003 combines our differentiated microdystrophin transgene with a next generation muscle-tropic capsid and a transient transfection manufacturing process that may help address the unmet needs for the Duchenne community." Jessie Hanrahan Ph.D. Chief Regulatory Officer added "We appreciate the FDA's review of the IND and look forward to continuing to work in collaboration with the agency when we initiate dosing of DMD patients."

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 and R17 nNos binding protein domains. Preclinical data suggests this may be important for both muscular function and durability of benefit in patients.

"IND clearance for SGT-003 is a critical step in bringing a potential next generation gene therapy to the clinic and making a meaningful impact on the lives of those living with Duchenne. We are working closely with clinical sites to dose the first participants, driven by the belief that better therapies are urgently required to treat this devastating disease," said Gabriel Brooks, M.D., Chief Medical Officer at Solid Biosciences.

Based on the clearance, Solid plans to move expeditiously to submit the study for IRB approval at the clinical trial sites and expects to commence patient screening shortly thereafter. The planned Phase 1/2 trial, SGT-003-101, is a first in human, open-label, multicenter trial to determine the safety and tolerability of SGT-003 in pediatric patients with DMD at a dose of 1E14vg/kg. SGT-003 will be administered as a one-time intravenous infusion to patients in two cohorts with a minimum of three patients each, with the potential for cohort expansion. Cohort 1 will study patients aged 4 to < 6 years of age with DMD. Long-term safety and efficacy will be evaluated for a total of 5 years following treatment.

In an mdx mouse model of Duchenne, SGT-003 demonstrated rapid transduction, showing robust microdystrophin expression levels in the heart, quadriceps, and diaphragm by day 4 post-gene therapy treatment. SGT-003 in nonhuman primates was shown to increase biodistribution to cardiac and skeletal muscle including the diaphragm versus AAV9. These studies suggest increased transgene expression and an improved safety profile compared to first generation microdystrophin gene therapies.

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 expectations for working closely with the FDA and clinical sites, initiating dosing, increasing durability and expression, improving safety, developing an innovative therapy and meeting unmet need; 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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