# Solid Biosciences Receives Rare Pediatric Disease Designation from the FDA for Duchenne Muscular Dystrophy Gene Therapy Candidate SGT-003

April 1, 2024

– SGT-003 Granted Rare Pediatric Disease, Orphan Drug and Fast Track Designations in U.S. –

– Site initiations scheduled for April; patient dosing expected to begin in Q2 2024 –

CHARLESTOWN, Mass., April 01, 2024 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company developing precision genetic medicines for neuromuscular and cardiac diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation for SGT-003, the company's next-generation Duchenne muscular dystrophy (Duchenne) gene therapy candidate.

"Solid's receipt of Rare Pediatric Disease Designation for SGT-003 highlights the continuing need for transformational treatments for this devastating disease," said Bo Cumbo, President and Chief Executive Officer at Solid Biosciences. "The key components of SGT-003 were rationally designed to improve on first generation gene therapies to provide skeletal muscle tropism, enhanced durability, and improved clinical outcomes. With site activation scheduled in April, and patient screening beginning shortly thereafter, we anticipate dosing patients in Q2 of this year."

The planned Phase 1/2 trial, INSPIRE Duchenne, 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 with DMD ages 4 to < 6 and cohort 2 will study patients with DMD ages 6 to < 8. We anticipate providing an initial safety update for the first three to four patients enrolled in the INSPIRE Duchenne trial in mid-2024, and we anticipate providing initial expression and functional data from those patients in the fourth quarter of 2024.

"Preclinical data suggests that SGT-003 has potential to significantly improve on existing treatments for Duchenne by using a muscle tropic proprietary capsid to deliver a DNA sequence encoding a shortened form of the dystrophin protein which, importantly, includes the nNOS binding domain. nNOS is believed to play a crucial role in both muscular function and endurance," said Dr. Gabriel Brooks, M.D., Chief Medical Officer at Solid Biosciences. "We look forward to rapidly bringing SGT-003 to the clinic and hope to all Duchenne patients in need."

**About Rare Pediatric Disease Designation**
Rare Pediatric Disease Designation is granted by the FDA for serious or life-threatening diseases in which manifestations primarily affect children ages 18 years and younger. In addition, the disease must affect fewer than 200,000 people in the United States. The program is designed to encourage development of new drugs for the prevention and treatment of rare pediatric diseases. If a new biologics license application (BLA) is approved for SGT-003, Solid may be eligible to receive a priority review voucher. This voucher could be redeemed to obtain priority review for any subsequent marketing application for a different product and may be sold or transferred.

**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 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 Solid Biosciences**
Solid Biosciences is a life sciences company focused on advancing a portfolio of gene therapy candidates 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, 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 initiating dosing and availability of clinical trial data and the potential benefits of SGT-003; the potential benefits of Rare Pediatric Disease Designation; 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 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 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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