Solid Biosciences Receives FDA Rare Pediatric Disease Designation for SGT-212 Dual Route of Administration Gene Therapy for Friedreich's Ataxia

December 1, 2025

– SGT-212 has been granted FDA Rare Pediatric Disease and Fast Track designations –

– SGT-212 is the only dual route gene therapy in development to treat Friedreich's ataxia –

– FALCON Phase 1b clinical trial participant screening underway –

CHARLESTOWN, Mass., Dec. 01, 2025 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) (the "Company" or "Solid"), a life sciences company developing precision genetic medicines for neuromuscular and cardiac diseases, today announced that it received Rare Pediatric Disease designation from the U.S. Food and Drug Administration (FDA) for SGT-212, the Company's investigational gene therapy for Friedreich's ataxia (FA). SGT-212 will deliver the full-length frataxin gene via dual routes of administration, utilizing both direct intradentate nucleus (IDN) and intravenous (IV) infusions, and was designed to promote restoration of therapeutic levels of the frataxin protein to address neurologic, cardiac and systemic clinical manifestations of FA.

Rare Pediatric Disease designation is granted by the FDA for serious and life-threatening diseases that primarily affect individuals under 18 years of age. The designation provides the Company with the potential to receive a pediatric priority review voucher (PRV), which can expedite the review for future Biologic License Applications. The PRV may be redeemed to receive priority review for another marketing application or may be sold or transferred.

Jessie Hanrahan, Ph.D., Chief Regulatory & Preclinical Operations Officer of Solid Biosciences, said, "Receiving Pediatric Rare Disease designation marks another significant milestone for our Friedreich's ataxia program, SGT-212. Together with the Fast Track designation granted earlier this year, it recognizes our dual-route clinical approach for FALCON, our first-in-human trial, which is now screening participants, as an important first step in meeting an unmet need for FA. These designations are designed to help accelerate time to market and enhance engagement with the FDA. We look forward to continued collaboration with regulators to bring this therapy to patients as quickly as possible."

About Rare Pediatric Disease Designation
The U.S. Food and Drug Administration Rare Pediatric Disease designation incentivizes development of therapies for serious or life-threatening rare pediatric diseases. Under this program, sponsors may qualify for a priority review voucher (PRV) upon approval that can be redeemed to receive priority review for a different product. The sponsor may also transfer or sell the voucher to another sponsor.

About SGT-212
SGT-212 is a recombinant AAV-based gene replacement therapy for Friedreich's ataxia (FA) designed to deliver full-length human frataxin (FXN) via a dual route of administration: intradentate nucleus (IDN) infusion, using an FDA-approved, stereotactic, precision MRI-guided device, followed by an intravenous (IV) infusion to increase therapeutic FXN levels in the cerebellar dentate nuclei and in cardiomyocytes, respectively. Targeted delivery to the dentate nuclei will be confirmed in real time via gadolinium, an MRI-enhancing contrast agent. Restoration of FXN levels is expected to repair the underlying mitochondrial dysfunction in neurons and cardiomyocytes to address neurologic, cardiac and systemic manifestations of the disease.

About Friedreich's Ataxia (FA)
FA is an inherited, life-threatening, degenerative multisystem disease caused by defects in the frataxin gene that disrupt production of the frataxin protein, a mitochondrial iron-binding protein involved in essential cellular processes, including energy production. FA is known to cause progressive nervous system damage, movement problems, and cardiac dysfunction, with cardiac complications identified as the primary cause of death. FA impacts approximately 5,000 people in the United States and 15,000 in Europe. There are currently no treatments that provide a cure or halt disease progression.

About Solid Biosciences
Solid Biosciences is a precision genetic medicine company focused on advancing a portfolio of gene therapy candidates targeting rare neuromuscular and cardiac diseases, including SGT-003 for Duchenne muscular dystrophy (Duchenne), SGT-212 for Friedreich's ataxia (FA), SGT-501 for catecholaminergic polymorphic ventricular tachycardia (CPVT), SGT-601 for TNNT2-mediated dilated cardiomyopathy and additional fatal, genetic cardiac diseases. The Company is also focused on developing innovative libraries of genetic regulators and other enabling technologies with promising potential to significantly impact gene therapy delivery cross-industry. Solid is advancing its diverse pipeline and delivery platform in the pursuit of uniting experts in science, technology, disease management, and care. Patient-focused and founded by those directly impacted by Duchenne, Solid's mission is to improve the daily lives of patients living with devastating rare 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 goals, priorities and key clinical and preclinical milestones; strategies and expectations for the company's SGT-003 and other programs, ; expectations for additional site activations, planned enrollment, planned regulatory interactions and the potential approval pathways for SGT-003; timing of planned clinical trials of SGT-003; t 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 advance SGT-003 and other programs, capsid libraries and other enabling technologies 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 and early-stage clinical trials of the company's product candidates; obtain, maintain or protect intellectual property rights related to its product

candidates; replicate preliminary or interim data from early-stage clinicals trials in the final data of such trials; compete successfully with other companies that are seeking to develop Duchenne, FA, CPVT 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 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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