Solid Biosciences Receives FDA Orphan Drug Designation for SGT-212 Dual-Route Gene Therapy for the Treatment of Friedreich's Ataxia

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- SGT-212 has received FDA Fast Track, Rare Pediatric Disease and Orphan Drug designations -

- Dosing of the first participant in the Phase 1b FALCON trial has been completed, with initial data expected in H2 2026, subject to participant enrollment -

CHARLESTOWN, Mass., Jan. 12, 2026 (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 U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to SGT-212 for the treatment of Friedreich's ataxia (FA). Additionally, earlier today, the Company reported that the first participant has been dosed in FALCON, a Phase 1b, first-in-human clinical trial evaluating SGT-212 for the treatment of FA.

"Receiving Orphan Drug, Fast Track and Rare Pediatric Disease designations underscores the significant unmet need the FA community faces and recognizes the therapeutic potential of SGT-212's novel, dual-route administration," said Jessie Hanrahan, Ph.D., Chief Regulatory & Preclinical Operations Officer of Solid Biosciences. "We anticipate that the ability to leverage these regulatory designations will help streamline and potentially accelerate SGT-212's development path, and we look forward to working closely with regulators to bring a potential new treatment option to the FA community."

SGT-212 is a first-in-class investigational gene therapy for the treatment of FA that employs a dual-route administration designed to target the neurologic, cardiac and systemic manifestations of the disease, which are key drivers of morbidity and mortality seen in FA. SGT-212 is delivered through a precise, stereotactic, MRI-guided intradentate nuclei (IDN) infusion to the cerebellar dentate nuclei followed by an intravenous (IV) infusion, with the goal of restoring therapeutic levels of frataxin.

About Orphan Drug Designation
The U.S. Food and Drug Administration (FDA) grants Orphan Drug designation (ODD) to investigational therapies intended for the treatment, diagnosis or prevention of rare diseases or conditions affecting fewer than 200,000 people in the United States. ODD provides certain development incentives, including tax credits for qualified clinical testing, waiver of FDA application fees and seven years of market exclusivity upon approval. These benefits are designed to encourage innovation and to potentially accelerate the availability of treatments for patients with serious underserved conditions.

About the FALCON Trial
FALCON is a first-in-human, open-label, multi-center Phase 1b clinical trial designed to evaluate the safety and tolerability of SGT-212 in participants aged 18-40 who have been diagnosed with FA and cardiac hypertrophy. FALCON is being conducted in the United States.

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 neurosurgical device in a stereotactic, precision MRI-guided technique, followed by an intravenous (IV) infusion, with the intent to increase therapeutic FXN levels in the cerebellar dentate nuclei, cardiomyocytes and other systemic tissues. Targeted delivery to the dentate nuclei will be confirmed in real time via MRI. 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 variants 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 neuromuscular and 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.

Cautionary Note Regarding 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; strategies and expectations for the company's SGT-212 program; timing of planned data announcements for the FALCON trial; anticipated benefits of SGT-212; 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, SGT-212, SGT-501, SGT-601 and other preclinical 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; enroll participants in ongoing trials; replicate preliminary or interim data from 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, SGT-212, SGT-501, SGT-601 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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