Solid Biosciences Receives FDA Fast Track Designation for SGT-212 Dual Route of Administration Gene Therapy for Friedreich's Ataxia

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- Only dual route gene transfer therapy in development to treat Friedreich's ataxia with FDA IND clearance and Fast Track designation -

CHARLESTOWN, Mass., Jan. 21, 2025 (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 Fast Track designation from the U.S. Food and Drug Administration (FDA) for SGT-212, the Company's, AAV-based gene therapy candidate for the treatment of Friedreich's ataxia (FA). SGT-212 will deliver the full-length frataxin gene via dual routes of administration incorporating intradentate nucleus (IDN) and intravenous (IV) infusions, designed to promote restoration of therapeutic levels of the frataxin protein to address neurologic, cardiac and systemic clinical manifestations of FA.

Fast Track designation is granted to products that are developed to treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs. This designation is intended to facilitate development and expedite review of qualifying drugs. SGT-212 will benefit from this designation by having more frequent interactions with the FDA and the potential to be eligible for priority review.

Bo Cumbo, President and CEO commented: "SGT-212 is the only FA therapy in development that is designed to address frataxin deficiency, the underlying cause of FA, and all manifestations of this devastating disease, and in doing so, hopefully halt the full spectrum of symptom progression regardless of where the patient is in their journey with this terrible disease. We believe Fast Track designation may enable us to more rapidly develop SGT-212 and bring hope to those living with FA who need and deserve better treatment options."

FDA IND clearance for SGT-212 was announced January 7th, 2025. The planned Phase 1b trial will be a first-in-human, open-label, multicenter study to evaluate the safety and tolerability of contemporaneous systemic IV and bilateral IDN administration of SGT-212 in adult non-ambulatory and ambulatory patients with FA. Dosing is expected to initiate in the second half of 2025 and participants in the trial will be followed for five years after receiving SGT-212.

"We are grateful for the FDA's recognition that the needs of the FA community remain underserved, and that SGT-212 has the potential to bring meaningful change to their lives," said Jessie Hanrahan, Ph.D., Chief Regulatory Officer at Solid Biosciences. "We look forward to working closely with the Agency to discuss the most effective and expeditious development pathway for SGT-212 to pursue future marketing authorization."

About Fast Track Designation
The FDA's Fast Track program facilitates the expedited development and review of new drugs that are intended to treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs.

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 the 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 Duchenne muscular dystrophy (Duchenne), Friedreich's ataxia (FA), catecholaminergic polymorphic ventricular tachycardia (CPVT), TNNT2-mediated dilated cardiomyopathy, BAG3-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 achieve key clinical milestones; the Company's pipeline of programs for neuromuscular and cardiac diseases, including its SGT-212 and SGT-003 programs and expectations for CTA filings, site activations, clinical development, initiation and enrollment in clinical trials, dosing, availability of clinical trial data and potential accelerated regulatory approval; the benefits and impact of Fast Track designation; 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-212, SGT-003, SGT-501, SGT-601, SGT-401 and other preclinical programs and capsid libraries on the timelines expected or at all; obtain and

maintain necessary approvals and designations 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; compete successfully with other companies that are seeking to develop Duchenne, Friedreich's ataxia 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-212, SGT-003, SGT-501, SGT-601, SGT-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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