Solid Biosciences Announces FDA IND Clearance for First-In-Industry Dual Route of Administration Gene Therapy to Treat Both Neurologic and Cardiac Manifestations of Friedreich's Ataxia

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- SGT-212 is the only full-length frataxin replacement gene therapy candidate targeting the CNS and cardiac manifestations of Friedreich's ataxia -

- Dual route of administration enables direct delivery of AAV-based gene therapy to the cerebellum and heart to potentially address the most significant symptoms of the disease -

- Phase 1b clinical trial initiation expected in 2H 2025 -

- Company to hold a conference call tomorrow, January 8, 2025, at 8:30 AM ET -

CHARLESTOWN, Mass., Jan. 07, 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 the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for SGT-212 for the treatment of Friedreich's ataxia (FA), a degenerative disease caused by insufficient levels of the frataxin protein. SGT-212 is the Company's novel, AAV-based FA gene therapy candidate designed to deliver full-length frataxin via systemic intravenous (IV) infusion as well as direct intradentate nuclei (IDN) infusion into the cerebellum. SGT-212 is designed to treat the neurologic and systemic clinical manifestations of FA to address the full spectrum of disease progression.

FA is a highly complex, multisystem disease that presents distinct challenges for drug development, in part because frataxin is a protein that requires: (1) precise expression levels to avoid fatal cardiac toxicities, and (2) on-target tissue localization in the cerebellum to achieve potential neurological clinical benefit. SGT-212 is the only candidate in development using two routes of administration to address the cardiac manifestations of FA while also directly delivering therapy to the dentate nuclei in the cerebellum, the region most affected and implicated in FA-associated neurologic decline.

Bo Cumbo, President and CEO of Solid Biosciences, commented: "SGT-212 has been intentionally designed to enable highly targeted delivery of our gene therapy to both the dentate nuclei and cardiac tissue. The IND was supported by a robust preclinical package demonstrating safe transduction and frataxin expression in these target tissues, with significant restoration of neurologic function and reversal of the cardiac implications of the disorder in mice. Over the years, we have tested several candidates using different methods of administration and have conducted multiple NHP studies, some of which extended out to a year. Based on this research, we believe a dual route of administration targeting multiple systems is the best approach in development to directly address the neurological implications that profoundly impact the everyday life of patients, while simultaneously targeting the cardiac manifestations that play a key role in more progressed disease. SGT-212 offers a truly differentiated approach to addressing FA with the potential to treat the full spectrum of symptoms, and we hope to meet each patient where they are in their FA disease course."

Jennifer Farmer, Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA), added: "We congratulate Solid Biosciences on reaching this significant milestone. Gene therapy approaches are aimed at the underlying causes of FA, and thus important in the overall strategy to treat and cure this disease. There has been encouraging progress in the FA treatment landscape; however, there is still unmet medical need for our patient community. Through our work with individuals living with FA and their families, we know they seek therapies designed to treat the debilitating neurologic symptoms that people living with FA face day-to-day, such as loss of ambulation and coordination, dysarthria, along with the life-shortening cardiac disease. SGT-212's unique, precision approach targets both the cerebellum and cardiac tissue using a dual route of administration, and in doing so, aims to address the underlying cause of the disease and the progression of FA. We look forward to continued partnership with the Company as they advance SGT-212 into the clinic later this year."

In the second half of 2025, the Company expects to initiate a first-in-human, open-label, dose-finding Phase 1b clinical trial of SGT-212. The study will enroll non-ambulatory and ambulatory adult patients living with FA across up to three cohorts and will evaluate the safety and tolerability of contemporaneous systemic and bilateral IDN administration of SGT-212. Participants in the trial will be followed out to five years after receiving SGT-212.

Mr. Cumbo continued: "We are thankful for our strong partnerships with FARA, the FA patient community, FA212 LLC led by Tom Hamilton, the University of Pennsylvania and James Wilson M.D. Ph.D., all of whom contributed to research and development leading to this IND clearance. We would also like to recognize the University of Florida for its commitment to FA and the tireless work conducted on our behalf in support of patients."

Conference Call
The Company will host a conference call tomorrow, January 8, 2025, at 8:30 AM ET to discuss the IND clearance for SGT-212 as well as other corporate updates. A live and archived webcast of the call will be available on Solid's website at www.solidbio.com under the "News & Events" tab in the Investor Relations section, or by clicking here.

Participants may also access the live call by dialing 877-407-2991 (toll-free) or 201-389-0925 (international).

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 MRI-guided device, followed by an intravenous (IV) infusion to increase therapeutic Fxn levels in the cerebellar dentate nuclei and in the cardiomyocytes, respectively. Restoration of Fxn levels is expected to repair the underlying mitochondrial dysfunction in neurons and cardiomyocytes to address both neurologic and cardiac manifestations of the disease.

SGT-212 was developed by FA212 LLC, a company founded by parents of children living with FA, the University of Pennsylvania, and Solid Biosciences.

University of Pennsylvania Financial Disclosure: The laboratory of Dr. Wilson at the University of Pennsylvania received sponsored research

funding from FA212 LLC. Penn owns an equity interest in FA212 LLC. Penn and Dr. Wilson have either received, or may receive in the future, financial consideration related to the licensing of certain Penn intellectual property to Solid Biosciences.

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 FARA
The Friedreich's Ataxia Research Alliance (FARA) is a national nonprofit organization dedicated to curing Friedreich's ataxia (FA) through research. FARA grants and activities provide support for basic and translational FA research, pharmaceutical/biotech drug development, clinical trials, and scientific conferences. FARA also serves as a catalyst, between the public and scientific community, to create worldwide exchanges of information that drive medical advances. For more information about FARA, visit www.curefa.org.

About Solid Biosciences
Solid Biosciences is a precision genetic medicine company focused on advancing a portfolio of gene therapy candidates including SGT-003 for the treatment of Duchenne muscular dystrophy (Duchenne), SGT-212 for the treatment of Friedreich's ataxia, SGT-501 for the treatment of catecholaminergic polymorphic ventricular tachycardia (CPVT), SGT-601 for the treatment of TNNT2-mediated dilated cardiomyopathy, SGT-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 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 approval; 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 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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