



Solid Biosciences Announces European Commission Orphan Designation for SGT-212 and Continued Progress in the FALCON Trial in Friedreich's Ataxia

October 26, 2026

- *Obtaining Orphan designation from the European Commission reflects Solid's commitment to the global development of SGT-212 for individuals living with Friedreich's ataxia -*
- *SGT-212 is a first-in-class dual route FA gene therapy designed to combine direct, MRI-guided delivery to the dentate nuclei with systemic delivery to reach cardiac and other systemic tissues -*
- *Five participants have been dosed in the ongoing Phase 1b FALCON clinical trial and SGT-212 has been well tolerated as of October 5, 2026 -*
- *Solid expects to report initial FALCON clinical data in Q1 2027 -*

CHARLESTOWN, Mass., Oct. 26, 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 the European Commission (EC), acting upon the positive opinion of the European Medicines Agency's Committee for Orphan Medicinal Products, has granted Orphan designation to SGT-212 for the treatment of Friedreich's ataxia (FA). Solid also announced that five participants have received SGT-212 in the Phase 1b FALCON clinical trial, with no treatment-related serious adverse events observed as of October 5, 2026.

SGT-212 is a novel investigational gene therapy specifically designed to address the multisystem biological impact of FA. Its dual-route administration combines precise, MRI-guided delivery directly to the cerebellar dentate nuclei with systemic intravenous delivery intended to reach the heart and other affected systemic tissues. Degeneration of the dentate nuclei, key sites of neurologic pathology in FA, disrupts coordinated movement and contributes to progressive loss of balance, limb control and oculomotor function as well as to bulbar dysfunction, including declines in speech and swallowing. Through this dual-route approach, SGT-212 is designed with the expectation of delivering the full-length frataxin gene to the tissues known to drive disease-specific morbidity and mortality, with the intent to restore mitochondrial health for patients living with FA.

"For families affected by Friedreich's ataxia, the need for new treatment options remains significant, and this designation supports the continued development of SGT-212 globally as we advance a therapy intentionally designed for the unique challenges of FA," said Jessie Hanrahan, Ph.D., Chief Regulatory & Preclinical Operations Officer at Solid Biosciences. "With five participants now dosed in the FALCON trial, we continue to build clinical experience with this first-in-class, dual-route approach and look forward to reporting initial clinical data in the first quarter of 2027."

In addition to the EC orphan designation, SGT-212 has been awarded Fast Track, Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration.

About Orphan Designation in the European Union

The EC grants orphan designation for medicinal products intended to treat a life-threatening or chronically debilitating disease that affects no more than five people in 10,000 in the EU, provided there is no other satisfactory treatment option or the medicine can be of significant benefit to those affected by a specific condition. Designated drugs are granted certain incentives including reduced regulatory fees, scientific and protocol assistance and market exclusivity for 10 years in the EU, if approved.

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 25,000 globally. There are currently no treatments that provide a cure or halt disease progression.

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 the FALCON Clinical 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 to 40 who have been diagnosed with FA. FALCON is being conducted in the United States.

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 and follow us on LinkedIn at [Solid Biosciences](https://www.linkedin.com/company/solid-biosciences) and X at [@Solid_Bio](https://twitter.com/Solid_Bio).

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; the ability to successfully achieve and execute on the company’s goals; anticipated benefits of SGT-212 and other clinical and preclinical programs and technologies; strategies and expectations for the company’s SGT-212 and other clinical and preclinical programs and technologies; expectations for planned site activation, planned enrollment, planned regulatory interactions and the potential approval pathways for SGT-212 and other clinical programs; the cash runway of the company and 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, 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; manufacture sufficient quantities of our drug product in a timely manner and maintain adequate supply to support our clinical development and potential commercialization; obtain, maintain or protect intellectual property rights related to its product candidates; replicate preliminary or interim data from clinical 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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