Solid Biosciences Announces Receipt of European Commission Orphan Drug Designation for SGT-003 for the Treatment of Duchenne Muscular Dystrophy

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- Orphan drug designation from the European Commission underscores Solid's commitment to advance SGT-003 through a global development effort for individuals living with Duchenne muscular dystrophy -

CHARLESTOWN, Mass., April 28, 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 from the European Medicines Agency Committee for Orphan Medicinal Products, has granted Orphan drug designation to SGT-003 for the treatment of Duchenne muscular dystrophy (Duchenne).

"This Orphan designation recognizes SGT-003's potential to address the significant unmet need in Duchenne muscular dystrophy and supports our firm commitment to advancing SGT-003 for patients globally," said Jessie Hanrahan, Ph.D., Chief Regulatory & Preclinical Operations Officer at Solid Biosciences. "This designation builds on the clinical and regulatory momentum for SGT-003 and complements our previously granted Innovation License and Access Pathway (ILAP) designation in the U.K., which enables early and enhanced engagement with regulators and other stakeholders as part of our broader international development efforts."

SGT-003 has received numerous regulatory designations from global health authorities, reflecting a coordinated, multinational development strategy with deep regulatory engagement across key geographies. In addition to the EC Orphan designation and the Innovation Passport designation under the new U.K. ILAP program, SGT-003 has also been awarded Fast Track, Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration.

SGT-003 is being evaluated in two ongoing clinical trials designed to support potential regulatory authorizations in multiple countries: INSPIRE DUCHENNE, a Phase 1/2 clinical trial, and IMPACT DUCHENNE, a Phase 3 randomized, double-blind, placebo-controlled clinical trial.

About Orphan Drug Designation in the European Union
The EC grants orphan drug 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 Duchenne
Duchenne is a genetic muscle-wasting disease predominantly affecting boys, with symptoms usually appearing between three and five years of age. Duchenne is a progressive, irreversible, and ultimately fatal disease that affects approximately one in every 5,000 live male births and has an estimated prevalence of 10,000 to 15,000 cases in the United States alone.

About SGT-003
SGT-003 is an investigational gene therapy containing a differentiated microdystrophin construct and a proprietary, next-generation capsid, POLARIS-101™ (formerly known as AAV-SLB101), which was rationally designed to target integrin receptors, and has shown enhanced cardiac and skeletal muscle transduction with decreased liver targeting in data from the Phase 1/2 INSPIRE DUCHENNE clinical trial and in nonclinical studies. SGT-003's microdystrophin construct uniquely includes the R16/17 domains, which localize nNOS to the muscle. Nonclinical studies have shown that nNOS can improve blood flow to the muscle thereby reducing muscle breakdown from ischemia and muscle fatigue. Together, these design features suggest that SGT-003 could be a potential best-in-class investigational gene therapy for the treatment of Duchenne.

About INSPIRE DUCHENNE
INSPIRE DUCHENNE is a first-in-human, open-label, single-dose, multicenter Phase 1/2 clinical trial to evaluate the safety, tolerability and efficacy of SGT-003 in pediatric participants with a genetically confirmed Duchenne diagnosis with a documented dystrophin gene mutation. INSPIRE DUCHENNE is a multinational trial designed to enroll participants in the United States, Canada, the United Kingdom and Italy.

About IMPACT DUCHENNE
IMPACT DUCHENNE is a Phase 3 randomized, double-blind, placebo-controlled trial to evaluate the efficacy of a single dose of SGT-003 in ambulatory participants aged 7 to less than 12 with a genetically confirmed Duchenne diagnosis. IMPACT DUCHENNE is a multinational trial intended to support potential regulatory authorizations.

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.

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-003; strategies and expectations for the company's SGT-003 program; expectations for planned enrollment, planned regulatory interactions and the potential approval pathways for SGT-003; 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 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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