Solid Biosciences Announces FDA IND and Health Canada CTA Approval for First-in-Class Cardiac Gene Therapy to Treat Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)

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- SGT-501 is a novel gene therapy for rare, life-threatening, genetic arrhythmogenic disease with no approved therapies -

- SGT-501 has received Orphan Drug Designation and Rare Pediatric Disease Designation from the FDA -

- Solid expects to initiate Phase 1b clinical trial of SGT-501 in the fourth quarter of 2025 -

- Expands Solid's clinical pipeline to include first cardiac indication with urgent unmet medical need -

CHARLESTOWN, Mass., July 08, 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 approval of its Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA) and clinical trial application (CTA) by Health Canada for SGT-501, a novel gene therapy approach for the treatment of catecholaminergic polymorphic ventricular tachycardia (CPVT), a highly malignant, arrhythmogenic channelopathy caused by genetic mutations that impact the ryanodine receptor (RYR2) in cardiac muscle. The Company expects to initiate a Phase 1b clinical trial to evaluate the safety, tolerability and efficacy of SGT-501 in the fourth quarter of 2025.

Gabriel Brooks, M.D., Chief Medical Officer of Solid, said: "Despite being identified nearly 50 years ago, CPVT still lacks FDA-approved therapies – this announcement reflects a critical development in the treatment of this underserved, often fatal, cardiac disease. SGT-501 offers a precision genetic approach targeting the underlying pathophysiology of the disease: abnormal calcium releases from the sarcoplasmic reticulum in an otherwise structurally sound heart. We believe SGT-501 has the unique potential to provide durable protection and may be capable of liberating patients from the ever-present threat of lethal arrhythmias and life-limiting prohibitions on exercise. Solid is proud to launch this program to help address this clear unmet need, further expanding our pipeline of differentiated and thoughtfully designed genetic medicines."

Silvia Priori, M.D., Ph.D., Professor of Cardiology at the University of Pavia and Director of the Molecular Cardiology Unit at the IRCCS Maugeri in Pavia, Italy, said: "After decades during which we, the clinical community, have been limited in our ability to treat people living with CPVT, I have long hoped for the day when a genetic-modifying therapy becomes available. We are proud to partner with Solid who advanced fundamental work from our labs at Maugeri, which demonstrated the principle that calsequestrin overexpression can have a therapeutic impact on multiple forms of CPVT in both cellular and mouse models of disease. This work was further developed into a compelling IND safety and efficacy package through close collaboration with, and studies conducted by, the Solid team. I look forward to clinical updates for SGT-501 and seeing how this potential medicine may benefit people living with CPVT who are in critical need of disease-specific medicines."

About SGT-501
SGT-501 is an AAV-based gene therapy candidate designed to deliver a functional, full length, codon-optimized copy of the human cardiac calsequestrin (CASQ2) gene to heart muscle cells. In the context of RYR2 variants, increasing CASQ2 protein levels enhances buffering of free calcium in the sarcoplasmic reticulum, stabilizing the RYR2, which results in reduced diastolic calcium leak into the cytosol. Stabilization of the RYR2 in its closed conformation supports the maintenance of normal cardiac rhythm with the potential to protect against ventricular tachycardia. SGT-501 received Orphan Drug Designation and Rare Pediatric Disease Designation from the U.S. FDA and has potential as a first-in-class therapy to correct the underlying RYR2 instability and calcium dysregulation causes of CPVT.

The AAV-CASQ2 gene therapy approach utilized by SGT-501 was pioneered by Dr. Silvia Priori and the IRCCS ICS Maugeri lab in Pavia, Italy. As a globally recognized leader in inherited arrhythmias, Dr. Priori has long been at the forefront of translational cardiac research. Solid Biosciences in-licensed this innovative therapeutic in 2023 and subsequently advanced it into IND-enabling preclinical development, marking an important step toward bringing this potential treatment to individuals living with CPVT.

About Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)
Catecholaminergic polymorphic ventricular tachycardia (CPVT) is a heart condition marked by abnormal rhythms; specifically, an abnormally fast heartbeat (ventricular tachycardia), which can be triggered by adrenergic stimulation such as physical activity or emotional stress. These arrhythmias can cause unexplained fainting, seizures, cardiac arrest and sudden death. CPVT is estimated to affect roughly 1 in 10,000 individuals globally, although it is often misdiagnosed, and is typically identified in children and young adults. It is mainly caused by mutations in the RYR2 and CASQ2 genes, which disrupt calcium regulation in heart muscle cells, impairing proper heart contraction and relaxation. There are currently no treatments that address the underlying mechanisms of CPVT.

To learn more about CPVT and SGT-501, watch a brief CPVT expert and patient video by clicking here.

About Istituti Clinici Scientifici Maugeri Spa SB (ICS Maugeri)
ICS Maugeri S.p.A. Società Benefit is Italy's leading healthcare provider specializing in rehabilitation and comprehensive care for vulnerable patients. It stands out for its strong focus on scientific research, operating 18 Clinical Institutes - including 9 recognized as IRCCS (Scientific Institutes for Research, Hospitalization and Healthcare) and 9 as Scientific Clinical Institutes -along with 6 outpatient centers, 1 Environmental Research Center, and a Poison Control Center and National Toxicology Information Center.

ICS Maugeri is involved not only in scientific research but also in the application of advanced technologies across medical disciplines, offering personalized care pathways and centers of excellence for specific conditions and clinical specialties. The Group employs more than 4,000 professionals, where medical and professional excellence is intrinsically linked to core values of equity, inclusivity, equality, and transparency - principles that have always guided Maugeri's mission.

Headquartered in Pavia, ICS Maugeri operates 23 healthcare facilities across 7 Italian regions, including 9 IRCCS, 10 Scientific Clinical Institutes, 5

outpatient clinics, a Poison Control Center –Toxicology Center, and an Environmental Research Center.

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-501, 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-501, SGT-212, SGT-003, 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-501, SGT-212, SGT-003, 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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