Solid Biosciences to Present at the American Society of Gene and Cell Therapy's 28th Annual Meeting

April 29, 2025

CHARLESTOWN, Mass., April 29, 2025 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company developing precision genetic medicines for neuromuscular and cardiac diseases, will present data from the Phase 1/2 INSPIRE DUCHENNE trial evaluating SGT-003, a next-generation gene therapy product candidate intended for the treatment of Duchenne muscular dystrophy (Duchenne), at the 28th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), from May 13-17, 2025, in New Orleans, LA.

"The data we will present at this year's ASGCT Annual Meeting reflect Solid's significant advances in next-generation capsid design and therapeutic approach for Duchenne, including the encouraging initial safety, expression, and biomarker data in the INSPIRE DUCHENNE clinical trial," said Bo Cumbo, President and CEO of Solid. "We are committed to accelerating safe and effective precision genetic medicines for individuals living with rare neuromuscular and cardiac diseases, and look forward to engaging with the medical and scientific community to advance the field of genetic medicine."

Oral Presentations

1. Title: Initial Experience from the INSPIRE DUCHENNE Phase I/II Study of SGT-003 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy

Session Title: Late-Breaking Abstracts II
Date/Time: May 17, 9:15 – 9:30am CT
Location/Room: Room 391-392
Presenter: Kevin Flanigan, MD, Advisor Consultant & Researcher, Nationwide Children's Hospital and investigator in the INSPIRE DUCHENNE clinical trial

2. Title: Insight into the Mechanism of Action of AAV-SLB101, a Novel Muscle-Tropic Capsid for Neuromuscular and Cardiac indications

Session Title: AAV Gene Transfer (C): Antibody Evasion, Cardiac & Neuromuscular Targets
Date/Time: May 17, 11:30 – 11:45am CT
Location/Room: New Orleans Theater A
Presenter: Jessica Boehler, PhD, Principal Scientist, Solid Biosciences

3. Title: Potential for AAV-SLB101-Mediated Gene Transfer Treatment in the Context of Natural Seropositivity and After an AAVrh74 Treatment

Session Type: Oral presentation
Session Title: Modulation of Humoral Immune Responses in AAV Gene Transfer
Date/Time: May 16, 1:30 – 3:15pm CT
Location/Room: Room 291-292
Presenter: Jessica Boehler, PhD, Principal Scientist, Solid Biosciences

Poster Presentations

4. Title: AAV-SLB101, a Novel Muscle-Tropic Capsid, Increases Gene Delivery and Expression Versus AAV9 and AAVrh74 in Mouse Models of DMD and FSHD Muscle Disease

Date/Time: May 14, 5:30 – 7:00pm CT
Location/Room: Poster Hall I2
Presenter: Prushti Bhavsar, Senior Associate Scientist II, Solid Biosciences

5. Title: Full Length Transgene Quantification Utilizing NanoMosaic Tessie Technology

Date/Time: May 13, 6:00 – 7:30pm CT
Location/Room: Poster Hall I2
Presenter: Isabella Pajevic, Senior Associate Scientist II, Solid Biosciences

6. Title: Capillary Isoelectric Focusing (cIEF) Platform for Characterization of Charge Variants of Adeno-Associated Virus (AAV) Capsids and Impact on Their Transduction Efficiency

Date/Time: May 13, 6:00 – 7:30pm CT
Location/Room: Poster Hall I2
Presenter: Brandon Hoyle, Principal Associate Scientist, Solid Biosciences

7. Title: Automation of AAV Capsid ELISA on Tecan Fluent

Date/Time: May 14, 5:30 – 7:00pm CT
Location/Room: Poster Hall I2
Presenter: Sarah Tong, Associate Scientist, Solid Biosciences

Following completion of the conference, presentations will be available on the Scientific Publications & Presentations page of the Our Science section of the Company website, or by clicking here.

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 3,500 to 5,000 live male births and has an estimated prevalence of 5,000 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, AAV-SLB101, which was rationally designed to target integrin receptors, and has shown enhanced cardiac and skeletal muscle transduction with decreased liver targeting 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 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 anticipated benefits of SGT-003; the Company's SGT-003 clinical program, including planned enrollment and site activations in the INSPIRE DUCHENNE trial, planned regulatory interactions and the potential accelerated approval pathway; 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, 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; replicate preliminary or interim data from early-stage clinicals trials in the final data of such trials; 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-003, SGT-212, 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.

Solid Biosciences Investor Contact:
Nicole Anderson
Director, Investor Relations and Corporate Communications
Solid Biosciences Inc.
investors@solidbio.com

Media Contact:
Glenn Silver
FINN Partners
glenn.silver@finnpartners.com

SOLID BIOSCIENCES

Source: Solid Biosciences Inc.