Solid Biosciences Provides Interim Positive Clinical Update on Phase 1/2 INSPIRE DUCHENNE Trial

March 11, 2026

- 40 participants have been dosed with SGT-003 in the INSPIRE DUCHENNE trial to date -

- Robust microdystrophin expression with consistent mechanistic evidence of dystrophin associated protein complex (DAPC) restoration and improvements in muscle integrity, including stabilization and improvement in cardiac function (LVEF) observed after treatment with SGT-003 -

- SGT-003 continued to demonstrate an encouraging safety profile and has been generally well tolerated in the 40 participants dosed as of March 11, 2026 -

- SGT-003 is administered using a lower-burden, steroid-only prophylactic immunomodulation regimen -

- The Company plans for additional meetings with the FDA in H1 2026 for guidance on a potential accelerated approval pathway for SGT-003 and expects to provide an update mid-2026 -

CHARLESTOWN, Mass., March 11, 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 reported updated positive interim data from the ongoing Phase 1/2 INSPIRE DUCHENNE clinical trial of SGT-003. The new data, including additional muscle biopsy, serum biomarker, cardiac function, and safety analyses, add to a growing dataset that suggests the potential of the biological activity of SGT-003 microdystrophin therapy. The interim update was delivered in an oral presentation at the 2026 MDA Annual Meeting on March 11, 2026, by Aravindhan Veerapandiyan, M.D., Director of the Comprehensive Neuromuscular Program and Co-Director of the Muscular Dystrophy Association Care Center at Arkansas Children’s Hospital, and principal investigator in the INSPIRE DUCHENNE trial.

Bo Cumbo, President and CEO of Solid Biosciences commented, “These data continue to suggest that SGT-003 may be having a disease-relevant treatment effect, with observed robust microdystrophin expression, restoration of key components of the dystrophin-associated protein complex suggesting a direct biologic correlate of dystrophin activity, and improvements across multiple biomarkers of muscle integrity. Together with the promising safety and tolerability profile, these interim results reinforce our confidence in the potential of SGT-003 to meaningfully impact the disease course of Duchenne. Duchenne continues to have an unmet medical need. We are pursuing guidance on a potential accelerated approval pathway for SGT-003 and look forward to continued engagement with the FDA as we work toward that goal. Our focus remains on urgently advancing SGT-003 to provide the Duchenne community with an additional therapeutic choice.”

Dr. Veerapandiyan commented, “What stands out in these data is the biological consistency observed across multiple independent measures of muscle structure, health, and preservation. This, combined with an encouraging safety and tolerability profile, is compelling. Solid Biosciences’ uniquely comprehensive quantitative biomarker analyses provide critical insights into SGT-003, and based on these findings, I believe this therapy has the potential to become an important treatment option for patients living with Duchenne.”

INSPIRE DUCHENNE Updated Interim Clinical Data

INSPIRE DUCHENNE is a Phase 1/2 first-in-human, open-label, single-dose, multicenter trial designed to evaluate the safety, tolerability and efficacy of SGT-003 in pediatric participants with Duchenne at a dose level of 1E14vg/kg. SGT-003 is administered as a one-time intravenous infusion. The interim clinical data reported in this release are as of a February 23, 2026, data cutoff date.

SGT-003 has been generally well tolerated in the 40 participants dosed as of March 11, 2026. The safety and tolerability profile observed in the INSPIRE DUCHENNE trial continued to be promising; SGT-003 is administered using a low-burden, steroid-only prophylactic immunomodulation regimen. The trial is being conducted at 15 clinical sites across the US, Canada, Italy and the United Kingdom and participant dosing remains ongoing.

Microdystrophin transduction and expression levels, beta-sarcoglycan localization and nNOS activity were evaluated by biopsy in 20 participants (ages 1-10 years) at Day 90 and in 3 participants at Day 360 (Table 1). Results demonstrated robust mean vector copies per nucleus and microdystrophin expression as well as properly localized and restored beta-sarcoglycan-positive fibers and nNOS activity-positive fibers. Beta-sarcoglycan and nNOS are critical components of the dystrophin-associated protein complex (DAPC). In Duchenne, the absence of dystrophin destabilizes the DAPC, triggering a cascade of structural, signaling and metabolic defects that impair muscle integrity. Reconstituting critical components of the DAPC, including beta-sarcoglycan and nNOS, could suggest biologic correlation of SGT-003’s treatment effect. Solid’s microdystrophin construct is the only microdystrophin gene therapy, approved or investigational, that contains the R16/R17 binding domain, which uniquely localizes nNOS to the muscle.

Table 1: 90- and 360-day biopsy results

	Day 90 (n=20 unless noted)	Day 360 (n=3)
Mean vector copies per nucleus	11	12
Mean microdystrophin expression by western blot (%)	60% (n=19)	91%
Mean microdystrophin expression by mass spectroscopy (%)	52% (n=17)	86%
Mean microdystrophin-positive fibers by immunofluorescence (%)	63%	69%
Properly localized and restored beta-sarcoglycan-positive fibers (%)	60%	69%
nNOS activity-positive fibers (%)	35%	33%

Western blot and mass spectrometry baselines were 0% mean normal dystrophin, microdystrophin-positive fibers by immunofluorescence was based on a manual count, as are beta-sarcoglycan and nNOS-positive fibers. These assays are conducted by multiple external vendors; at the time of analysis, one western blot sample and three mass spectrometry samples had not been received.

Additionally, Solid has identified an extensive biomarker panel to comprehensively evaluate treatment effect on muscle integrity. Collectively, these observed biomarker improvements at Day 90 and Day 360 suggest improved muscle fiber health and stability, reduced ongoing muscle damage, and an interruption of the chronic degeneration/regeneration cycle that is characteristic of Duchenne.

In particular, embryonic myosin heavy chain (eMHC) is an informative predictor of disease progression. eMHC is typically expressed during fetal development but is also expressed when muscle satellite cells differentiate into muscle fibers, which in Duchenne occurs in response to muscle fiber damage. In the absence of functional dystrophin, newly generated muscle fibers also fail, leading to continued but ultimately futile satellite cell activation. A mean 44% observed reduction in eMHC positive fibers seen at Day 90 (n=20) suggests that SGT-003 treatment has potentially disrupted this chronic and futile degeneration-regeneration cycle, stabilizing muscle fibers and preserving the reservoir of satellite cells.

Table 2: Improvements in multiple biomarkers of muscle integrity observed at Day 90 and Day 360

Serum Biomarkers	Day 90 Mean Reductions (n=24 unless noted)	Day 360 Mean Reductions (n=7 unless noted)
Serum creatine kinase (CK)	38%	37%
Serum alanine transaminase (ALT)	43%	27%
Serum aspartate aminotransferase (AST)	30%	32%
Serum lactate dehydrogenase (LDH)	46% (n=21)	38% (n=6)
Serum titin	22% (n=11)	25% (n=2)
Certain data from a subset of participants were not available at the time of analysis.		

While cardiac assessments were initially included as safety evaluations, stabilization-to-improvement in systolic function continues to be observed as of the data cutoff date, as measured by left ventricular ejection fraction (LVEF). Observed improvements were driven largely by participants with low-normal baseline LVEF (defined as ≤ 60%[1]). Cardiomyopathy is a leading cause of death in Duchenne, with 25% of individuals displaying evidence of cardiomyopathy by six years of age, increasing to 59% by 10 years of age.[2]

A presentation summarizing the interim data update can be accessed on the Presentations page of the Investors section of the Company's website.

SGT-003 Regulatory Status

As announced on February 9, 2026, Solid has reached alignment with the FDA on the overall study design for the Company's randomized, double-blind, placebo-controlled Phase 3 clinical trial of SGT-003, IMPACT DUCHENNE. The FDA agreed that the trial design was reasonable including: the patient population of ambulant participants 7 to <12 years of age, the primary endpoint of change from baseline in Time to Rise (TTR) velocity from supine position evaluated at 18 months and other key secondary endpoints. Participant screening is underway and the Company anticipates dosing the first participant in the IMPACT DUCHENNE trial in the first quarter of 2026.

The Company plans to have additional meetings with the FDA in the first half of 2026 to receive guidance on a potential accelerated approval pathway for SGT-003 and expects to provide additional regulatory and clinical updates mid-2026.

References

1. Romanowicz J, et al. *J Am Soc Echocardiogr.* 2023;36(3):310-323.
2. Gandhi S, et al. *Cells*. 2024;13(14):1168.

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 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, 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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