Solid Biosciences Reports Second Quarter 2025 Financial Results and Provides Business Updates

August 12, 2025

*- **Duchenne (SGT-003):** 15 participants dosed in the Phase 1/2 INSPIRE DUCHENNE trial and dosing remains ongoing; On track to discuss regulatory pathways with U.S. FDA in Q4 2025 -*

*- **FA (SGT-212):** Phase 1b trial initiation expected in Q4 2025 -*

*- **CPVT (SGT-501):** Phase 1b trial initiation expected in Q4 2025 -*

*- **Capsids (AAV-SLB101):** Over 25 agreements or licenses with academic labs, institutions and corporations for the use of AAV-SLB101 have been executed -*

*- **Cash:** Company ended Q2 2025 with $268.1 million in cash, cash equivalents and available-for-sale securities; Solid has anticipated cash runway into H1 2027 -*

CHARLESTOWN, Mass., Aug. 12, 2025 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) (the "Company" or "Solid"), a life sciences company developing precision, next generation, genetic medicines for neuromuscular and cardiac diseases, today reported financial results for the second quarter ended June 30, 2025, and provided a business update.

Bo Cumbo, President and CEO of Solid, commented: "With three clinical-stage programs for neuromuscular and cardiac diseases, Solid now stands at the forefront of innovation in genetic medicine. The strategic expansion of our pipeline beyond Duchenne to include differentiated therapies for both FA and CPVT marks a meaningful stride forward that reflects both our disciplined execution and steadfast dedication to the patient communities we serve.

"We continue to receive overwhelming interest in the INSPIRE DUCHENNE trial from families and the clinical community and now have 10 active clinical sites and more than 20 additional participants identified to potentially enter the INSPIRE DUCHENNE trial. As of August 12, 2025, we have dosed 15 participants and SGT-003 continues to be well tolerated, with no treatment emergent SAEs observed, and we continue to recruit and dose patients. We look forward to FDA discussions later this year and will continue to work diligently to advance a new gene therapy option for the Duchenne community," Mr. Cumbo concluded.

Company Updates

Neuromuscular Pipeline
SGT-003 for Duchenne Muscular Dystrophy (Duchenne)

- As of August 12, 2025, 15 participants have been dosed in the INSPIRE DUCHENNE trial with participant dosing ongoing across multiple cohorts; SGT-003 continues to be well tolerated with no treatment emergent SAEs observed and an immune suppression regimen that consists of steroids alone. As of August 12, 2025, we have observed only one (N=1/15), Grade 1, Adverse Event of Special Interest (AESI) of elevated liver enzymes post treatment that occurred during the steroid taper period; this patient has shown no clinical manifestations.
- The Company expects to dose a minimum of 20 participants by year-end.
    - In addition to the 15 participants dosed as of August 12, 2025, more than 20 additional patients have been screened, pre-screened or identified by clinical trial sites as potential participants in the INSPIRE DUCHENNE trial.
- The INSPIRE DUCHENNE trial now has 10 active sites across the U.S., Canada, Italy and the United Kingdom, with additional sites expected to be activated by year-end.
- The Company expects to initiate a separate randomized, double-blind, placebo-controlled trial evaluating SGT-003 outside of the United States in the fourth quarter of 2025, with the aim of supporting potential global regulatory authorizations.
- The Company expects to meet with the U.S. Food and Drug Administration (FDA) in the fourth quarter of this year to discuss regulatory pathways for SGT-003.

SGT-212 for Friedreich's Ataxia (FA)

- The Company expects to initiate a first-in-human, open-label, Phase 1b clinical trial of

SGT-212 in the fourth quarter of 2025. The trial is expected to enroll non-ambulatory and ambulatory adult participants living with FA in up to three cohorts and is designed to evaluate the safety and tolerability of systemic and bilateral IDN administration of SGT-212.
- SGT-212 is the first investigational gene therapy for FA to utilize a dual route of administration and is intended to promote restoration of therapeutic levels of the frataxin protein to address the neurologic, cardiac and systemic clinical manifestations of FA.

Cardiac Pipeline
SGT-501 for Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)

- As announced on July 8, 2025, the U.S. FDA cleared the Investigational New Drug (IND) application and Health Canada approved the Clinical Trial Application (CTA) for SGT-501, a first-in-class, investigational gene therapy for the treatment of CPVT, a life-threatening, genetic arrhythmogenic disease with no approved therapies.
- As announced on July 23, 2025, SGT-501 received Fast Track designation from the FDA. SGT-501 previously received Orphan Drug and Rare Pediatric Disease designations from the FDA.
- SGT-501 is a novel gene therapy intended to promote excess levels of the cardiac CASQ2 protein to address the underlying ryanodine receptor (RYR2) instability and calcium dysregulation seen in CPVT.
- The Company expects to initiate a Phase 1b clinical trial in adult participants with CPVT to evaluate the safety, tolerability and efficacy of SGT-501 in the fourth quarter of 2025.

SGT-601 for TNNT2 Thin Filament Cardiomyopathy

- SGT-601 is a novel gene therapy designed to treat cardiomyopathy caused by pathogenic variants in the TNNT2 gene, which codes for the cardiac troponin T protein. SGT-601 leverages AAV-SLB101 capsid delivery of the full-length, wild type TNNT2 transgene, with the aim of regulating cardiac muscle contraction and relaxation.
- IND-enabling preclinical work is underway, with proof-of-concept efficacy studies demonstrating that SGT-601 treatment elicited robust, cardiac-selective expression of TNNT2, resulting in improvements in survival, cardiac structure and function at multiple dose levels in a translationally relevant mouse model.
- There are no FDA-approved therapies that address the underlying cause of TNNT2-related cardiomyopathy.

Platform Technologies – Capsids & Other
Capsids & Promoters

- AAV-SLB101 is the Company's proprietary capsid used in SGT-003, which has been well tolerated as of August 12, 2025 (N=15), in the Phase 1/2 INSPIRE DUCHENNE trial. In the first three participants dosed, for which 90-day biopsy biomarker data were initially reported in February 2025, rapid and robust levels of vector transduction, microdystrophin expression, and reduced liver targeting were observed.
- Solid currently has agreements and licenses with over 25 academic labs, institutions and corporations for the use of AAV-SLB101, with additional agreements and licenses expected to be executed over the coming quarters.
- The Company is building multiple cardiac and neuromuscular next-generation capsid and promoter libraries with final capsid selection from the first cardiac capsid library anticipated in the fourth quarter of 2025.

Second Quarter 2025 Financial Highlights

- **Cash Position:** Solid had $268.1 million in cash, cash equivalents, and available-for-sale securities as of June 30, 2025, compared to $148.9 million as of December 31, 2024. The Company expects that its cash, cash equivalents, and available-for-sale securities as of June 30, 2025, will enable it to fund its operational runway into the first half of 2027.

- **Research and Development (R&D) Expenses:** R&D expenses for the second quarter of 2025 were $32.4 million, compared to $19.5 million for the second quarter of 2024. The increase of $13.0 million in research and development expenses was primarily due to a $9.9 million increase in costs for SGT-003 primarily related to manufacturing, regulatory, and clinical costs, a $2.3 million increase in personnel related expenses, a $2.1 million increase in costs for SGT-601 primarily related to manufacturing costs, a $1.0 million increase in costs for SGT-212 primarily related to clinical and research costs, partially offset by a $1.5 million decrease in costs for other development programs primarily related to lower research costs, and a $1.2 million decrease in costs for SGT-501 primarily related to lower research and manufacturing costs.

- **General and Administrative (G&A) Expenses:** G&A expenses for the second quarter of 2025 were $9.3 million, compared to $8.3 million for the second quarter of 2024. The increase of $1.0 million was primarily related to a $1.2 million increase in personnel related costs, partially offset by a $0.3 million decrease in consulting fees.

- **Net Loss:** Net loss for the second quarter of 2025 was $39.5 million, compared to $25.1 million for the second quarter of 2024.

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 key clinical and preclinical milestones; strategies and expectations for the company's SGT-003, SGT-212, SGT-501 and SGT-601 programs; expectations for additional CTA filings, site activations, planned enrollment, planned regulatory interactions and the potential approval pathways for SGT-003; timing of planned clinical trials of SGT-212 and SGT-501; 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; obtain, maintain or protect intellectual property rights related to its product candidates; replicate preliminary or interim data from early-stage 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.

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 INC.
SELECTED FINANCIAL INFORMATION (UNAUDITED)

CONDENSED CONSOLIDATED BALANCE SHEETS **(in thousands, except share data)**	**June 30, 2025**	**December 31, 2024**
Cash and cash equivalents	$ 138,933	$ 80,235
Available-for-sale securities	129,177	68,685
Prepaid expenses and other current assets	10,530	8,382
Operating lease, right-of-use assets	23,133	24,295
Property and equipment, net	4,317	4,747
Other non-current assets	278	366
Restricted cash	1,924	1,952
Total Assets	$ 308,292	$ 188,662
Accounts payable	$ 4,704	$ 4,237
Accrued expenses and other current liabilities	16,546	19,852
Operating lease liabilities	1,943	1,787
Finance lease liabilities	953	1,231
Derivative liabilities	5,700	3,150
Operating lease liabilities, excluding current portion	20,155	21,159
Total stockholders' equity	258,291	137,246
Total Liabilities and Stockholders' Equity	$ 308,292	$ 188,662
Common stock outstanding	77,602,741	40,468,141

CONDENSED CONSOLIDATED STATEMENT OF OPERATIONS	**Three Months Ended June 30,**		**Six Months Ended June 30,**	
(in thousands, except per share data)	**2025**	**2024**	**2025**	**2024**
Operating expenses:				
Research and development	$ 32,415	$ 19,461	$ 63,329	$ 38,334
General and administrative	9,278	8,327	18,416	16,316
Total operating expenses	41,693	27,788	81,745	54,650
Loss from operations	(41,693)	(27,788)	(81,745)	(54,650)
Other income, net:				
Interest income	2,966	2,565	5,266	5,216
Interest expense	(60)	(88)	(128)	(183)
Change in fair value of derivative liabilities	(900)	—	(2,550)	—
Other income, net	207	239	395	242
Total other income, net	2,213	2,716	2,983	5,275
Net loss	$ (39,480)	$ (25,072)	$ (78,762)	$ (49,375)
Net loss per share, basic and diluted	$ (0.42)	$ (0.61)	$ (0.98)	$ (1.25)
Weighted average shares of common stock outstanding, basic and diluted	94,140,286	40,934,361	80,317,588	39,544,867

Source: Solid Biosciences Inc.