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

May 15, 2025

*- **Duchenne (SGT-003)**: Participant dosing ongoing in the Phase 1/2 INSPIRE DUCHENNE trial; Solid on track to discuss accelerated pathways with U.S. FDA later in 2025 -*

*- **FA (SGT-212):** Dosing of first participant anticipated in the second half of 2025 -*

*- **CPVT (SGT-501):** FDA IND on track for submission first half of 2025 -*

*- **Cash:** Company ended first quarter 2025 with $306.9 million in cash, cash equivalents, investments and available-for-sale securities; Solid has anticipated cash runway into the first half of 2027 -*

CHARLESTOWN, Mass., May 15, 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 first quarter ended March 31, 2025, and provided a business update.

Bo Cumbo, President and CEO of Solid, commented: "The positive initial three patient, 90-day data in the INSPIRE DUCHENNE trial of SGT-003, our next-generation, investigational gene therapy to treat Duchenne muscular dystrophy, were a milestone in the development of a meaningful treatment candidate for this terrible disease. With the initial data demonstrating robust microdystrophin expression and improvements across biomarkers of muscle integrity and, excitingly, encouraging biomarkers of cardiac and liver health, we continue to dose participants as we prepare to engage with the FDA later this year on the potential for accelerated pathways.

"To our patient communities, we know that safety is a critical factor in choosing any medical therapy. SGT-003, utilizing our proprietary, rationally designed capsid, AAV-SLB101, contains the lowest dose of any Duchenne gene therapy currently marketed or in clinical development, and to date, has demonstrated an encouraging safety and tolerability profile. We look forward to the 28th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) where, on Saturday, we will provide an interim safety update from the INSPIRE DUCHENNE trial during our late-breaking oral presentation.

"As an organization, we are intensely focused on driving innovation and advancing the C&GT field with the aspiration to make gene therapies safer, more effective, and truly accessible. These efforts originate with our differentiated and thoughtfully designed neuromuscular and cardiac therapeutic pipeline and expand to the development and broad out-licensing of next-generation delivery technologies, beginning with AAV-SLB101. We look forward to executing across these objectives over the coming quarters to drive meaningful value for all of our stakeholders," Mr. Cumbo concluded.

Company Updates

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

- As announced on February 18, 2025, the Company reported positive initial clinical data from the first three participants dosed in the Phase 1/2 INSPIRE DUCHENNE trial.
  - SGT-003 has been well tolerated in all participants dosed to date, with no treatment emergent serious adverse events (SAEs) or AEs of acute liver injury observed.
- The INSPIRE DUCHENNE trial continues dosing participants across multiple cohorts, and the Company expects to dose approximately 20 total participants by year-end.
- The INSPIRE DUCHENNE trial now has eight active sites in the U.S., Canada and Italy, with additional sites expected to be activated by year-end.
- The Company plans to meet with the FDA later this year to share safety and efficacy results from additional treated participants in the INSPIRE DUCHENNE study and to discuss accelerated regulatory pathways. The Company expects the meeting to occur in the fourth quarter of 2025.

SGT-212 for Friedreich's Ataxia (FA)

- As announced on January 7, 2025, the FDA has cleared the IND for SGT-212 for the treatment of FA. SGT-212 is the first gene therapy candidate for FA to utilize a dual route of administration that was designed to promote restoration of therapeutic levels of the frataxin protein to address neurologic, cardiac and systemic clinical manifestations of FA.

- The Company expects to initiate a first-in-human, open-label, Phase 1b clinical trial of SGT-212 in the second half of 2025. The trial is expected to enroll non-ambulatory and ambulatory adult participants living with FA across up to three cohorts and is designed to evaluate the safety and tolerability of systemic and bilateral IDN administration of SGT-212.

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

- IND-enabling Good Laboratory Practice (GLP) toxicology studies of SGT-501 in non-human primates were completed in the first quarter of 2025.
- The Company anticipates submitting an IND for SGT-501 for the treatment of CPVT in 1H 2025.

Platform Technologies – Capsids & Other
Capsids & Promoters

- 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.
- AAV-SLB101 is the Company's proprietary capsid used in SGT-003, which in the initial three participants dosed in the INSPIRE DUCHENNE study demonstrated rapid and robust levels of vector transduction, microdystrophin expression, and reduced liver targeting.
- Solid currently has partnership agreements with 19 academic labs, institutions and corporations for the use of AAV-SLB101, with additional partnerships expected to be executed over the coming quarters.

First Quarter 2025 Financial Highlights

- **Cash Position:** Solid had $306.9 million in cash, cash equivalents, and available-for-sale securities as of March 31, 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 March 31, 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 first quarter of 2025 were $30.9 million, compared to $18.9 million for the first quarter of 2024. The increase of $12.0 million was primarily due to a $7.4 million increase in costs for SGT-003 primarily related to manufacturing, regulatory, and clinical costs, a $1.9 million increase in personnel-related expenses, a $1.3 million increase in other development program expenses primarily due to manufacturing costs, a $0.8 million increase in costs for SGT-212 primarily related to regulatory and clinical costs, and a $0.7 million increase in costs for SGT-501 primarily related to research, regulatory, and clinical costs.
- **General and Administrative (G&A) Expenses:** G&A expenses for the first quarter of 2025 were $9.1 million, compared to $8.0 million for the first quarter of 2024. The increase of $1.1 million was primarily due to a $1.7 million increase in personnel-related costs, partially offset by a $0.4 million decrease in other G&A expenses.
- **Net Loss:** Net loss for the first quarter of 2025 was $39.3 million, compared to $24.3 million for the first 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 milestones; the company's SGT-003 and SGT-212 programs, including expectations for additional CTA filings, site activations, planned enrollment, planned regulatory interactions and the potential accelerated approval pathway for SGT-003; the company's expectations for submission of INDs; 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.

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SOLID BIOSCIENCES INC.
SELECTED FINANCIAL INFORMATION (UNAUDITED)

CONDENSED CONSOLIDATED BALANCE SHEETS **(in thousands, except share data)**	**March 31, 2025**	**December 31, 2024**
Cash and cash equivalents	$ 210,629	$ 80,235
Available-for-sale securities	96,289	68,685
Prepaid expenses and other current assets	6,491	8,382
Operating lease, right-of-use assets	23,720	24,295
Property and equipment, net	4,483	4,747
Other non-current assets	319	366
Restricted cash	1,973	1,952
Total Assets	$ 343,904	$ 188,662
Accounts payable	$ 6,439	$ 4,237
Accrued expenses and other current liabilities	14,576	19,852
Operating lease liabilities	1,868	1,787
Finance lease liabilities	1,096	1,231
Derivative liabilities	4,800	3,150
Operating lease liabilities, excluding current portion	20,662	21,159
Total stockholders' equity	294,463	137,246
Total Liabilities and Stockholders' Equity	$ 343,904	$ 188,662
Common stock outstanding	77,492,959	40,468,141

CONDENSED CONSOLIDATED STATEMENT OF OPERATIONS **(in thousands, except per share data)**	**Three Months Ended March 31,** **2025**	**2024**
Operating expenses:		
Research and development	$ 30,914	$ 18,873
General and administrative	9,138	7,989
Total operating expenses	40,052	26,862
Loss from operations	(40,052)	(26,862)
Other income, net:		
Interest income	2,300	2,651

Interest expense	(68)	(95)
Change in fair value of derivative liabilities	(1,650)	—
Other income, net	188	3
Total other income, net	770	2,559
Net loss	$ (39,282)	$ (24,303)
Net loss per share, basic and diluted	$ (0.59)	$ (0.64)
Weighted average shares of common stock outstanding, basic and diluted	66,341,305	38,155,373

This press release was published by a CLEAR® Verified individual.

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