Solid Biosciences Reports Fourth Quarter and Full Year 2024 Financial Results and Provides Business Updates

March 6, 2025

*- **Duchenne**: Reported positive initial data from INSPIRE DUCHENNE trial of next-generation Duchenne gene therapy candidate, SGT-003; In mid-2025, Company plans to request a U.S. Food and Drug Administration (FDA) meeting to discuss potential accelerated approval pathways -*

*- **FA:** FDA Investigational New Drug (IND) clearance for first-in-human clinical study of SGT-212 utilizing a dual route of administration to treat Friedreich's ataxia (FA); Dosing of first participant anticipated in the second half of 2025 -*

*- **CPVT:** IND for SGT-501 on track for first half 2025 submission -*

*- **Cash:** Company ended 2024 with $148.9 million in cash, cash equivalents, investments and available-for-sale securities; Combined with gross proceeds from $200.0 million underwritten offering in February 2025, Solid has anticipated cash runway into the first half of 2027 -*

CHARLESTOWN, Mass., March 06, 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 fourth quarter and full year ended December 31, 2024, and provided a business update.

Bo Cumbo, President and CEO of Solid, commented: "The diligent execution of our Duchenne and FA development programs over the last 18 months has transformed Solid and given us tremendous momentum into 2025 and beyond. The initial 90-day data from the first three participants in the Phase 1/2 INSPIRE DUCHENNE trial of our next-generation Duchenne muscular dystrophy (Duchenne) program, SGT-003, while early, have shown a reassuring safety experience and promising improvements in biomarkers of muscle integrity and health. We believe these early indicators support the potential of SGT-003 to be a best-in-class gene therapy candidate to treat Duchenne. We understand the excitement around these data in the Duchenne patient communities and we are committed to moving with purpose and urgency to bring SGT-003 through the clinic as quickly as possible.

"The INSPIRE DUCHENNE data also provided a critical, first-in-human evaluation of our proprietary capsid, AAV-SLB101. We were very pleased to see that this capsid was well tolerated in the first six participants and has translated into highly robust transduction and expression levels. We anticipate these data should open up many more opportunities to use AAV-SLB101 in other clinical settings and indications, through external partnerships as well as our internal development programs, and we are excited for the strong potential there. We currently have partnership agreements with 19 academic labs, institutions and corporations for the use of AAV-SLB101, and are looking forward to adding more in the coming quarters.

"Additionally, in the second half of 2025, we anticipate initiating a first-in-human clinical trial evaluating SGT-212, our second active neuromuscular gene therapy program to receive FDA IND clearance. SGT-212 is our uniquely designed FA gene therapy candidate, which is the first gene therapy with IND clearance using a dual route of administration to address both the neurological and cardiac aspects of this devastating disease.

"Thanks to the support of our new and existing investors who participated in our recent $200.0 million equity offering, we are now positioned to accelerate toward exciting clinical milestones across our portfolio. The proceeds from the offering allow us to further invest in the advancement of SGT-003, SGT-212, SGT-501 and the rest of our promising development pipeline as we make strides towards bringing next-generation precision genetic medicines to those who need them most," Mr. Cumbo concluded.

Company Updates

Neuromuscular Pipeline
SGT-003 Next-Generation Duchenne muscular dystrophy (Duchenne) Program

- 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.
  - Observed high vector genome copies per nucleus, robust microdystrophin expression and early improvements in additional measures of muscle integrity, including:
    - Mean vector copies per nucleus: 18.7 (N=3),
    - Mean microdystrophin expression: 110% (N=3), as measured by western blot,
    - Mean microdystrophin expression: 108% (N=3), as measured by mass spectrometry,
    - Mean percent dystrophin positive fibers: 78% (N=3), as measured by immunofluorescence,
    - Mean beta sarcoglycan percent positive fibers: 70% (N=3),
    - Mean nNOS (neuronal nitric oxide synthase) percent positive fibers: 42% (N=3),
    - Improvements in 7 additional muscle integrity biomarkers (N=3), and
    - Early mean improvement in left ventricular ejection fraction (LVEF) of 8% from

baseline at Day 180 (N=2).
    - SGT-003 has been well tolerated in the 6 participants dosed as of the data cutoff date of February 11, 2025, with no serious adverse events (SAEs), suspected unexpected serious adverse reactions (SUSARs), or AEs of acute liver injury observed.
    - Enrollment in the trial is ongoing, and the Company expects to dose more than 10 total participants by early second quarter 2025, and approximately 20 total participants by the fourth quarter of 2025.
    - In mid-2025, the Company plans to request a meeting with the FDA to discuss potential accelerated approval pathways for SGT-003.

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 to utilize a dual route of administration to treat 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 concurrent 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.

SGT-601 for TNNT2-Mediated Dilated Cardiomyopathy

- Preclinical IND-enabling studies are underway and planned throughout 2025, with anticipated IND submission in the second half of 2026.

Mayo Clinic Collaboration

- As announced on December 4, 2024, Solid entered into a collaboration with Mayo Clinic to develop an AAV gene therapy platform for the development of therapies to treat sudden cardiac death-predisposing genetic cardiomyopathies and channelopathies.
- Under the collaboration, Solid received an exclusive license to their Suppression and Replacement ("Sup-Rep") gene therapy platform and an exclusive license to develop and commercialize six undisclosed cardiac gene therapy programs.
- These programs will use Solid's next-generation AAV capsids, including AAV-SLB101, and advanced manufacturing capabilities.

Platform Technologies – Capsids & Other
Solid is building an innovative library of enabling technologies across:

Capsids & Promoters

- The Company is building multiple cardiac and neuromuscular capsid and promoter libraries with final capsid selection from the first cardiac capsid library anticipated in the fourth quarter of 2025.
- The initial data reported from the INSPIRE DUCHENNE trial were a first-in-human evaluation of AAV-SLB101, Solid's proprietary capsid utilized in SGT-003, which has been well tolerated

in the first six participants dosed as of the data cutoff date of February 11, 2025.

Immunomodulation

- Further evaluation is expected to determine the viability of potential future dosing with AAV-SLB101 in AAV-gene therapy pre-treated and seropositive individuals in Duchenne.

CMC Purity

- Improvements in full-to-empty capsid ratios seen at research scales in Solid's early-stage cardiac programs.
- Current full-to-empty capsid ratios are approximately 80% full capsids for SGT-003 (Duchenne), approximately 85% full capsids for SGT-501 (CPVT) and approximately 92% full capsids for SGT-601 (TNNT2).[1]

Fourth Quarter and Full-Year 2024 Financial Highlights

- **Cash Position:** Solid had $148.9 million in cash, cash equivalents, and available-for-sale securities as of December 31, 2024, compared to $123.6 million as of December 31, 2023. When combined with the gross proceeds from Solid's $200.0 million February 2025 underwritten offering, the Company expects that its cash, cash equivalents, investments and available-for-sale securities as of December 31, 2024, will enable it to fund its operations into the first half of 2027.

- **Research and Development (R&D) Expenses:** R&D expenses for the fourth quarter of 2024 were $30.8 million, compared to $15.5 million for the fourth quarter of 2023. Research and development expenses for the full year ended December 31, 2024, were $96.4 million, compared to $76.6 million for the full year ended December 31, 2023. The increase of $19.9 million was primarily due to a $14.0 million increase in costs for SGT-501 from increased manufacturing and study related costs, a $4.6 million increase in costs for SGT-212 related to the entry into the asset purchase agreement with FA212 LLC, and a $6.7 million increase in license fees and research and consulting costs for other development programs, partially offset by a $5.7 million decrease in costs for SGT-003 related to manufacturing and study related costs.

- **General and Administrative (G&A) Expenses:** G&A expenses for the fourth quarter of 2024 were $9.1 million, compared to $6.8 million for the fourth quarter of 2023. General and administrative expenses for the full year ended December 31, 2024, were $33.3 million, compared to $27.8 million for the full year ended December 31, 2023. The increase of $5.5 million was primarily related to a $4.1 million increase in personnel costs.

- **Net Loss:** Net loss for the fourth quarter of 2024 was $42.6 million, compared to $20.3 million for the fourth quarter of 2023. Net loss for the full year ended December 31, 2024, was $124.7 million, compared to $96.0 million for the full year ended December 31, 2023.

References

[1]SGT-003 Good Manufacturing Practice (GMP) scale currently at 1,000L, SGT-501 GMP scale currently at 500L, and SGT-601 currently at 2L scale in process development.

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 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; 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	December 31,	
(in thousands, except share data)	**2024**	**2023**
Cash and cash equivalents	$ 80,235	$ 74,015
Available-for-sale securities	68,685	49,625
Prepaid expenses and other current assets	8,382	6,094
Operating lease, right-of-use assets	24,295	26,539
Property and equipment, net	4,747	6,624
Other non-current assets	366	209
Restricted cash	1,952	1,833
Total Assets	$ 188,662	$ 164,939
Accounts payable	$ 4,237	$ 2,032
Accrued expenses and other current liabilities	19,852	10,161
Operating lease liabilities	1,787	1,855
Finance lease liabilities	1,231	469
Derivative liabilities	3,150	—
Operating lease liabilities, excluding current portion	21,159	22,707
Finance lease liabilities, excluding current portion	—	1,234
Total stockholders' equity	137,246	126,481
Total Liabilities and Stockholders' Equity	$ 188,662	$ 164,939
Common stock outstanding	40,468	20,387

CONDENSED CONSOLIDATED STATEMENT OF OPERATIONS	Three Months Ended December 31,		Year Ended December 31,	
(in thousands, except per share data)	**2024**	**2023**	**2024**	**2023**
Operating expenses:				
Research and development	$ 30,770	$ 15,453	$ 96,431	$ 76,563
General and administrative	9,126	6,812	33,297	27,752
Restructuring charges	—	—	—	(63)
Total operating expenses	39,896	22,265	129,728	104,252

Loss from operations	(39,896)	(22,265)	(129,728)	(104,252)
Other income, net:				
Interest income	1,926	1,659	9,469	7,582
Interest expense	(75)	—	(340)	(440)
Change in fair value of derivative liabilities	(4,750)	—	(4,750)	—
Other income, net	198	270	652	1,095
Total other income, net	(2,701)	1,929	5,031	8,237
Net loss	$ (42,597)	$ (20,336)	$ (124,697)	$ (96,015)
Net loss per share, basic and diluted	$ (1.00)	$ (1.01)	$ (3.06)	$ (4.83)
Weighted average shares of common stock outstanding, basic and diluted	42,706,077	20,230,697	40,816,694	19,884,007

Source: Solid Biosciences Inc.