Solid Biosciences Reports Fourth Quarter and Full-Year 2021 Financial Results and 2-Year Efficacy and Safety Data from the Ongoing Phase I/II IGNITE DMD Clinical Trial of SGT-001

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- 2-year data from first three patients in the high dose (2E14 vg/kg) cohort suggest sustained or improved motor function, pulmonary function and clinically validated patient-reported outcomes compared with expected declines reported by natural history data -

- 90-day biopsy data from most recently dosed patients show microdystrophin expression that is consistent with earlier high dose (2E14vg/kg) cohort patients -

- Company to present updated IGNITE DMD data at the 2022 Muscular Dystrophy Association Clinical & Scientific Conference -

- Company advances SGT-001 clinical and regulatory strategies toward End of Phase II FDA discussions; Pipeline expansion activities continue for next generation Duchenne program, SGT-003; Company ends 2021 with $207.8 million in cash and investments -

CAMBRIDGE, Mass., March 14, 2022 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today provided an update from the ongoing IGNITE DMD Phase I/II clinical trial, as well as a business and financial update. The company reported positive two-year efficacy and safety data from the first three patients treated in the high dose (2E14 vg/kg) cohort of the ongoing Phase I/II IGNITE DMD clinical trial. Additionally, the company reported 90-day biopsy results for Patients 7-9, the most recent three patients treated in the high dose cohort.

Roxana Donisa Dreghici, MD, Senior Vice President, Head of Clinical Development at Solid will present “IGNITE DMD Phase I/II Study of SGT-001 Microdystrophin Gene Therapy for DMD: 2-Year Outcomes Update” in an oral session at the 2022 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference on Wednesday, March 16. The conference is taking place March 13-16 in-person in Nashville and virtually.

“We are encouraged by the motor function, pulmonary function and patient reported outcome data which show that patients continue to experience durable benefit across all reported measures two years after SGT-001 dosing,” said Dr. Dreghici. “This is important as the evidence suggests that these patients treated with SGT-001 show potentially sustained or improved performance at a time when expected natural history would suggest a decline. We are especially pleased that the data suggests sustained benefit of pulmonary function at the two year timepoint, which may lead to delayed ventilation and improved quality of life, as well as potentially an increased life expectancy.”

SGT-001 Update

Efficacy and Safety Data

Today, the company reported two-year interim efficacy and safety data from the first three patients treated with SGT-001 in the high dose (2E14 vg/kg) cohort as part of IGNITE DMD. Results suggest durable benefit 24-months post-administration of SGT-001, when compared to natural history. These data are consistent with results reported at the 12-month and 18-month time periods for the same patients. The average age of Patients 4-6 at the two year timepoint was 10.4 years.

Data from Patients 4-6 suggest improved motor function at two years post-infusion, as assessed by 6-Minute Walk Test and North Star Ambulatory Assessment, against expected natural history declines. In addition, the data suggest improved pulmonary function, as measured by forced vital capacity and peak expiratory flow, and sustained or improved patient reported outcome measures as assessed in key functional domains of the Pediatric Outcomes Data Collection Instrument (PODCI) when compared to both baseline and natural history.

The following table summarizes the interim efficacy results of Patients 4-6 at 24-months post-dosing. Data are presented as mean change from baseline and as the mean change from natural history at the 24-month timepoint. Mean difference from natural history is calculated as the difference between mean change from baseline for Patients 4-6 at 24-months and the expected changes from baseline over the same period, based on published natural history studies:

Summary of 24-Month Efficacy Results of IGNITE DMD for Patients 4-6 (2E14 vg/kg cohort)			
	Mean Age at Baseline (Range)	**Mean Change from Baseline (Range)**	**Mean Difference vs Natural History**
6-Minute Walk Test (meters)	8.4 years (6.8 to 10.7 years)	+16.0 (-12 to 39)	+100.6[1]
North Star Ambulatory Assessment (units)		-1.7 (-3 to -1)	+4.3[2]
Forced Vital Capacity (%p)		+9.2 (-1.4 to 29.0)	+19.2[3]
Peak Expiratory Flow (%p)		+6.5 (-5.8 to 14.8)	+16.5[4]
PODCI Global Function (points)		+6.3 (0 to 13)	+16.4[5]
PODCI Transfer/Basic Mobility (points)		+0.7 (-4 to 6)	+20.6[6]
PODCI Sports/Physical Functioning (points)		+13.3 (12 to 15)	+19.5[7]

1: -84.6m expected decline in 24 months after age 7 (Mercuri et al 2016)
2: -6.0 unit expected decline in 24 months after age 6.3 (Muntoni et al 2019)

3: -10.0%p expected decline in 24 months after age 6 (Mayer et al 2015)
4: -10.0%p expected decline in 24 months after age 6 (Mayer et al 2015)
5: -10.1 point expected decline in 24 months (Henricson et al 2013)
6: -19.9 point expected decline in 24 months (Henricson et al 2013)
7: -6.2 point expected decline in 24 months (Henricson et al 2013)

90-Day Biopsy Data
In addition, today, the company reported data from skeletal muscle biopsies collected three months after infusion of SGT-001 from the most recently dosed Patients 7-9. The range by immunofluorescence of 1% to 50% and by western blot of Below the 5% Limit of Quantification (BLQ) to 6.8%, were within the range of previously dosed Patients 4-6 in the high dose cohort. Microdystrophin expression levels for all six patients dosed in the high dose cohort (Patients 4-9) ranged from 1 to 70% by immunofluorescence and BLQ to 17.5% by western blot. All six patients dosed with SGT-001 in the high dose cohort have demonstrated microdystrophin expression and proper membrane localization.

Safety Findings
No new drug-related safety findings have been identified in Patients 1-9 in post-dosing periods of 90 days to approximately four years. We continue to follow dosed patients and collect data to support the potential benefit of SGT-001.

Regulatory Path Forward
As previously disclosed, Solid is planning to conclude dosing patients as part of IGNITE DMD this year and begin End of Phase II discussions with the Food and Drug Administration (FDA).

SGT-003 Update
Preclinical activities for Solid's next-generation pipeline program, SGT-003, progress with IND-enabling studies for SGT-003 started earlier in 2022. The company expects an Investigational New Drug (IND) application submission early 2023. In preclinical studies, SGT-003 has demonstrated improved biodistribution compared with AAV9 in various in vitro and in vivo models, with increased delivery to and expression in skeletal and heart muscle and reduced tropism for liver cells.

Financial Highlights
Collaboration revenue for the fourth quarter of 2021 was $3.2 million, compared to no collaboration revenue for the fourth quarter of 2020. Collaboration revenue for the full year ended December 31, 2021 were $13.6 million, compared to no collaboration revenue for the full year ended December 31, 2020. The increase in collaboration revenue is related to research services and cost reimbursement from our Collaboration Agreement with Ultragenyx, which the Company entered into in the fourth quarter of 2020.

Research and development expenses for the fourth quarter of 2021 were $14.6 million, compared to $15.7 million for the fourth quarter of 2020. Research and development expenses for the full year ended December 31, 2021 were $58.7 million, compared to $64.9 million for the full year ended December 31, 2020.

General and administrative expenses for the fourth quarter of 2021 were $7.2 million, compared to $5.6 million for the fourth quarter of 2020. General and administrative expenses for the full year ended December 31, 2021 were $27.1 million, compared to $21.6 million for the full year ended December 31, 2020.

Net loss for the fourth quarter of 2021 was $18.6 million, compared to $21.4 million for the fourth quarter of 2020. Net loss for the full year ended December 31, 2021 was $72.2 million, compared to $88.3 million for the full year ended December 31, 2020.

Solid had $207.8 million in cash, cash equivalents and available-for-sale securities as of December 31, 2021. The company expects that its cash, cash equivalents and available-for-sale securities will enable Solid to fund its operations and capital expenditures into the third quarter of 2023.

About SGT-001
Solid's SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy designed to address the underlying genetic cause of Duchenne. Duchenne is caused by mutations in the dystrophin gene that result in the absence or near absence of dystrophin protein. SGT-001 is a systemically administered candidate that delivers a synthetic dystrophin gene, called microdystrophin, to the body. This microdystrophin encodes for a functional protein surrogate that is expressed in muscles and stabilizes essential associated proteins, including neuronal nitric oxide synthase (nNOS). Data from Solid's clinical program suggests that SGT-001 has the potential to slow or stop the progression of Duchenne, regardless of genetic mutation or disease stage.

SGT-001 is based on pioneering research in dystrophin biology by Dr. Jeffrey Chamberlain of the University of Washington and Dr. Dongsheng Duan of the University of Missouri. SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, and Fast Track Designation in the United States and Orphan Drug Designations in both the United States and European Union.

About SGT-003
SGT-003, Solid's next-generation gene therapy candidate for the treatment of Duchenne, utilizes a rationally designed AAV-based vector to deliver the proprietary and differentiated microdystrophin construct that is also incorporated into SGT-001. SGT-003 has demonstrated improved biodistribution compared with AAV9 in various *in vitro* and *in vivo* models, with increased delivery to and expression in skeletal and heart muscle and reduced tropism for liver cells. Solid is targeting an IND filing in early 2023.

About Solid Biosciences
Solid Biosciences is a life sciences company focused on advancing transformative treatments to improve the lives of patients living with Duchenne. Disease-focused and founded by a family directly impacted by Duchenne, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation that causes Duchenne with our lead gene therapy candidate, SGT-001, as well as our recently announced next-generation gene therapy candidate, SGT-003. 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 the ability of the Company to continue dosing patients in the IGNITE DMD trial, the Company's plans to present data from IGNITE DMD, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 in patients with DMD, the Company's

regulatory plans, the Company's SGT-003 program, including the Company's expectation for filing an IND, timelines, 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 continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; obtain and maintain the necessary approvals from investigational review boards at IGNITE DMD clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll additional patients in IGNITE DMD and on the timeline expected; the Company's dosing strategy; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; whether the interim data referenced in this release will be predicative of the final results of the trial or will demonstrate a safe or effective treatment benefit of SGT-001; whether the methodologies, assumptions and applications the Company utilizes to assess particular safety or efficacy parameters will yield meaningful statistical results; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully optimize and scale its manufacturing process; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, SGT-003 and other product 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.

Investor Contact:
David Carey
FINN Partners
212-867-1768
David.Carey@finnpartners.com

Caitlin Lowie
Solid Biosciences
607-423-3219
clowie@solidbio.com

Media Contact:
Erich Sandoval
FINN Partners
917-497-2867
Erich.Sandoval@finnpartners.com

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