



Solid Biosciences Reports Fourth Quarter and Fiscal Year 2018 Financial Results and Provides Business Update

March 13, 2019

–Activities underway to commence dosing of second cohort of patients in the IGNITE DMD clinical trial at 2E14 vg/kg of SGT-001–

CAMBRIDGE, Mass., March 13, 2019 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (NASDAQ: SLDB) today reported financial results for the fourth quarter and fiscal year ended December 31, 2018 and provided a business update.

"In our first year as a public company, we have significantly advanced our innovative science for Duchenne muscular dystrophy, made meaningful progress towards commercial scale manufacturing and continued to add talent and capabilities to our team to prepare to bring differentiated therapies to patients," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "We are especially pleased to have received rapid approval to begin evaluating a higher dose of our investigational microdystrophin gene therapy, SGT-001, in the IGNITE DMD clinical trial. We look forward to continuing to understand its full potential in the clinic and to providing additional data from the study later this year."

"We are encouraged to continue advancing SGT-001 in the clinic," said Jorge Quiroz, M.D., Chief Medical Officer of Solid Biosciences. "Based on our preclinical data, we expect that increased doses of SGT-001 will achieve higher microdystrophin expression and, with agreement from the study's Data Safety Monitoring Board and the University of Florida Institutional Review Board, we have initiated the appropriate activities to dose escalate."

Recent Developments

- Today Solid announced that it has completed the necessary steps to escalate the dose of SGT-001 to 2E14 vg/kg in a second cohort of patients, which include agreement from the IGNITE DMD clinical trial Data Safety Monitoring Board (DSMB) and University of Florida Institutional Review Board (IRB). Solid will continue to enroll children in the study, which is a randomized, controlled, open-label, single-ascending dose Phase I/II clinical trial to assess the safety and efficacy of SGT-001 (an investigational AAV-mediated microdystrophin gene transfer) for the treatment of Duchenne muscular dystrophy (DMD), and intends to resume dosing adolescents in the future. Solid anticipates providing additional data on IGNITE DMD later this year.
- In February, Solid announced preliminary findings based on three-month biopsy data from the first three patients dosed with 5E13 vg/kg of SGT-001, the initial dose outlined in the IGNITE DMD protocol. In one patient, microdystrophin was detected via western blot below the five percent level of quantification of the assay and in approximately 10 percent of fibers via immunofluorescence. There were also signs of co-localization of neuronal nitric oxide synthase (nNOS) and beta-sarcoglycan associated with microdystrophin expression. In the second and third patients, microdystrophin was detected via immunofluorescence at very low levels, but it was undetectable via western blot. The safety profile of SGT-001 remains unchanged.
- In February, Solid's Scientific Advisory Board Chair Dr. Jeffery Chamberlain and other researchers at the University of Washington published preclinical data highlighting the unique attributes of the SGT-001 transgene in the journal *Molecular Therapy*. The paper, called *Development of Novel Micro-dystrophins With Enhanced Functionality*, supports the potential of the SGT-001 transgene to protect against contraction-induced injury and recruit key proteins of the dystrophin glycoprotein complex, including nNOS.
- In January, Solid presented additional information about its scalable manufacturing process at the 37th Annual J.P. Morgan Healthcare Conference, noting that the Company has successfully produced multiple batches at 250 liters in suspension, each of which can dose multiple patients, at good manufacturing practice-compliant facilities.
- In January, the Company appointed Lynette Herscha as Chief Legal Officer. Ms. Herscha brings to Solid more than 20 years of legal experience in the technology and biopharma industry. Prior to joining Solid, she served as General Counsel, Secretary and a member of the Executive Team at Concert Pharmaceuticals, Inc. Before joining Concert Pharmaceuticals, Ms. Herscha held various senior legal positions at Momenta Pharmaceuticals, Inc. and Phase Forward, Inc. She began her career at the law offices of Fullbright & Jaworski.

Financial Highlights

Research and development expenses for the fourth quarter of 2018 were \$17.8 million, compared to \$11.9 million for the prior year period. Research and development expenses for the year ended December 31, 2018 were \$58.0 million, compared to \$39.9 million for the year ended December 31,

2017. The increases were primarily attributed to an increase in research and development personnel and related facility costs, increased manufacturing costs and an increase in clinical development costs for SGT-001. These increases were offset by a reduction in preclinical costs associated with SGT-001.

General and administrative expenses for the fourth quarter of 2018 were \$4.6 million, compared to \$3.2 million for the prior year period. General and administrative expenses for the year ended December 31, 2018 were \$17.7 million, compared to \$15.0 million for the year ended December 31, 2017. The increases were primarily attributed to increased personnel and related facility costs, as well as other corporate expenses associated with being a public company.

Net loss for the fourth quarter of 2018 was \$21.9 million, compared to \$14.5 million for the fourth quarter of 2017. Net loss for the year ended December 31, 2018 was \$74.8 million, compared to \$53.2 million for the year ended December 31, 2017.

Solid had \$122.5 million in cash, cash equivalents and available-for-sale securities as of December 31, 2018, compared to \$69.1 million as of December 31, 2017. The increase was primarily the result of the completion of the Company's initial public offering on January 30, 2018.

Upcoming Conference

Management is scheduled to present at the Alliance of Regenerative Medicine (ARM) Cell & Gene Therapy Investor Day on Thursday, March 21, 2019 at 9:25 am ET in New York City.

A live video webcast will be available at <http://arminvestorday.com/webcast/> and will also be published there shortly after the event.

About SGT-001

Solid's lead candidate, SGT-001, is a novel adeno-associated viral (AAV) vector-mediated gene transfer under investigation for its ability to address the underlying genetic cause of Duchenne muscular dystrophy (DMD), 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 transgene, 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). SGT-001 utilizes AAV9, which has an affinity for muscle and is currently being evaluated in multiple clinical programs in other indications. Data from Solid's preclinical program suggest that SGT-001 has the potential to slow or stop the progression of DMD, 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 Solid Biosciences

Solid Biosciences is a life science company focused solely on finding meaningful therapies for Duchenne muscular dystrophy (DMD). Founded by those touched by the disease, Solid is a center of excellence for DMD, bringing together experts in science, technology and care to drive forward a portfolio of candidates that have life-changing potential. Solid is progressing programs across four scientific platforms: Corrective Therapies, Disease-Modifying Therapies, Disease Understanding and Assistive Devices. 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 our expectations regarding the IGNITE DMD clinical trial, and the potential of SGT-001, the sufficiency of our cash, cash equivalents and investments to fund our operation and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," 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 Solid's ability to obtain and maintain necessary approvals from the FDA and other regulatory authorities and investigational review boards at clinical trial sites; enroll patients in its clinical trials; continue to advance SGT-001 in clinical trials, including to proceed with dose escalation of IGNITE DMD; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully 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 DMD treatments and gene therapies; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. 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 Inc.
Consolidated Statements of Operations
(unaudited, in thousands, except share and per share data)

	Year Ended December 31,		
	2018	2017	2016
Revenue	\$ -	\$ -	\$ -

Operating expenses:			
Research and development	57,965	39,905	20,116
General and administrative	17,722	14,952	5,460
Total operating expenses	75,687	54,857	25,576
Loss from operations	(75,687)	(54,857)	(25,576)
Other income (expense):			
Revaluation of preferred unit tranche rights	-	459	1,163
Interest income	619	219	369
Other income	270	1,001	271
Total other income (expense), net	889	1,679	1,803
Net loss	\$ (74,798)	\$ (53,178)	\$ (23,773)
Net loss attributable to non-controlling interest	-	(1,060)	(2,234)
Net loss attributable to Solid Biosciences Inc.	\$ (74,798)	\$ (52,118)	\$ (21,539)
Decretion (accretion) of preferred units to redemption value	-	(959)	4,309
Redemption of preferred units	-	15,685	-
Redemption of redeemable interest from non-controlling interest in Solid GT	-	(1,925)	-
Net loss attributable to common stockholders	\$ (74,798)	\$ (39,317)	\$ (17,230)
Net loss per share attributable to common stockholders, basic and diluted	\$ (2.25)	\$ (2.88)	\$ (10.14)
Weighted average shares of common stock outstanding, basic and diluted	33,262,597	13,649,485	1,698,904

Solid Biosciences Inc.
Consolidated Balance Sheets
(unaudited, in thousands, except share and per share data)

	December 31,	
	2018	2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 86,366	\$ 52,080
Available-for-sale securities	36,098	17,014
Prepaid expenses and other current assets	6,175	1,499
Restricted cash	-	65
Total current assets	128,639	70,658
Property and equipment, net	10,422	2,429
Other non-current assets	209	-
Restricted cash	327	-
Deferred offering costs	-	3,106
Total assets	\$ 139,597	\$ 76,193
Liabilities, Preferred Units and Stockholders' / Members' Equity / (Deficit)		
Current liabilities:		
Accounts payable	\$ 3,691	\$ 5,066
Accrued expenses	8,235	5,972
Current portion of capital lease obligations	173	-
Other current liabilities	382	233
Total current liabilities	12,481	11,271
Capital lease obligations, net of current portion	859	-
Other non-current liabilities	1,074	-
Total liabilities	14,414	11,271
Series 2 Senior Preferred Units	-	55,002
Series 1 Senior Preferred Units	-	25,000
Junior Preferred Units	-	44,177
Stockholders' / Members' Equity / (Deficit)		
Series A, B, C and D Common Units	-	65,014
Common Stock	35	-
Additional paid-in capital	324,209	-
Accumulated other comprehensive loss	(5)	(13)
Accumulated deficit	(199,056)	(124,258)
Total stockholders' / members' equity (deficit)	125,183	(59,257)
Total liabilities, preferred units and stockholders' / members' equity	\$ 139,597	\$ 76,193

Media Contact:

Kate Niazi-Sai
+1 617-337-4680
media@solidbio.com

Investor Contact:

Geoffrey M. Grande, CFA
+1 617-766-3398
investors@solidbio.com



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