



Solid Biosciences Reports Third Quarter 2018 Financial Results And Provides Business Update

November 13, 2018

Two Additional Patients Dosed With SGT-001 Gene Transfer in IGNITE DMD Clinical Trial

Company Plans to Report Preliminary Results from IGNITE DMD in the First Quarter of 2019

CAMBRIDGE, Mass., Nov. 13, 2018 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (NASDAQ: SLDB) today reported financial results for the third quarter ended September 30, 2018 and provided a business update.

"We are pleased to have made significant progress toward our goal of bringing meaningful treatments to patients with Duchenne muscular dystrophy," said Ilan Ganot, Chief Executive Officer, Co-Founder and President of Solid Biosciences. "Most notably, we resumed dosing in the Phase I/II IGNITE DMD clinical trial for our SGT-001 microdystrophin gene therapy program. This progress was complemented by continued work on our innovative and scalable manufacturing process, which enabled us to move forward with the study as planned and without delay. We believe that the unique attributes included in SGT-001 could translate to significant benefit for patients and now look forward to providing preliminary biopsy data from IGNITE DMD in the first quarter of 2019. We also remain on track to provide data from our previously communicated interim analysis in the second half of 2019."

"We are pleased with the progress we have made on IGNITE DMD, dosing two additional patients with SGT-001 since the study resumed," said Jorge Quiroz, M.D., Chief Medical Officer of Solid Biosciences. "Six patients have now been randomized in IGNITE DMD, three to the active treatment group, all of whom are doing well, and three to the delayed treatment control group. Continuing to enroll IGNITE DMD is a top priority, and we look forward to understanding the potential of SGT-001 in the clinic. As always, we remain grateful to the patients and families participating in IGNITE DMD and the team at the University of Florida."

Recent Developments

- Solid is continuing to enroll IGNITE DMD, the Company's randomized, controlled Phase I/II clinical trial to assess the safety and efficacy of SGT-001 (AAV-mediated microdystrophin gene transfer) for the treatment of Duchenne muscular dystrophy (DMD). In total, six patients have been randomized in IGNITE DMD, three to the active treatment group and three to the delayed treatment control group. This number includes two additional patients who have been dosed since the study resumed in June. All three patients who have received SGT-001 are currently doing well.

There were no serious adverse events observed in the second or third patients dosed. Laboratory findings, including a transient decline in platelet count that has fully resolved, were quickly identified and managed per the study protocol. Solid continues to enroll patients in IGNITE DMD and plans to communicate preliminary results, including microdystrophin expression data, in the first quarter of 2019.

- In September, Solid announced that the Company appointed clinical and gene therapy expert Sukumar Nagendran, M.D., to its Board of Directors. Dr. Nagendran brings to Solid more than 30 years of experience in key functional areas, including gene therapy development, clinical strategy, medical affairs and diagnostics. Most recently, Dr. Nagendran was Chief Medical Officer & Senior Vice President of AveXis Inc., where he was responsible for overseeing and driving all clinical development and medical affairs strategy, notably for the company's late-stage AAV-mediated gene therapy program for Spinal Muscular Atrophy (SMA).
- In October, Solid received Fast Track Designation for SGT-001 from the U.S. Food and Drug Administration (FDA). The Fast Track program is designed to expedite the development and review of drugs to treat serious or life-threatening conditions and fill an unmet medical need.

Financial Highlights

Solid Biosciences reported a net loss of \$19.0 million for the third quarter of 2018 as compared to \$13.5 million for the third quarter of 2017. The net

loss was due to research and development expenses, as well as investments in the Company's infrastructure.

Research and development expenses for the third quarter of 2018 were \$14.7 million as compared to \$10.6 million for the prior year period. This increase was primarily due to personnel and facility related expenses and costs related to clinical development and manufacturing activities for SGT-001, as well as our other product candidates. These increases were offset by a reduction in preclinical costs associated with SGT-001.

General and administrative expenses were \$4.5 million for the third quarter of 2018 as compared to \$3.1 million for the prior year period. This increase was primarily due to personnel and facility related costs, as well as other corporate expenses.

Solid ended the third quarter of 2018 with \$145.4 million in cash, cash equivalents and available-for-sale securities as 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.

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 Solid's IGNITE DMD clinical trial, its anticipated achievement of milestones and the potential of SGT-001. 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; replicate in later 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; 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.
Condensed Consolidated Statements of Operations
(unaudited, in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	14,680	10,619	40,203	27,959
General and administrative	4,470	3,077	13,098	11,737
Total operating expenses	<u>19,150</u>	<u>13,696</u>	<u>53,301</u>	<u>39,696</u>
Loss from operations	<u>(19,150)</u>	<u>(13,696)</u>	<u>(53,301)</u>	<u>(39,696)</u>
Other income (expense):				
Revaluation of preferred unit tranche rights	-	(88)	-	(68)
Interest income	92	51	237	165
Other income	38	228	187	908
Total other income (expense), net	<u>130</u>	<u>191</u>	<u>424</u>	<u>1,005</u>

Net loss	(19,020)	(13,505)	(52,877)	(38,691)
Net loss attributable to non-controlling interest	-	-	-	(1,060)
Net loss attributable to Solid Biosciences Inc.	(19,020)	(13,505)	(52,877)	(37,631)
Accretion of preferred units to redemption value	-	-	-	(959)
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	<u>\$ (19,020)</u>	<u>\$ (13,505)</u>	<u>\$ (52,877)</u>	<u>\$ (24,830)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.55)</u>	<u>\$ (0.79)</u>	<u>\$ (1.61)</u>	<u>\$ (1.99)</u>
Weighted average shares of common stock outstanding, basic and diluted	<u>34,539,001</u>	<u>17,096,280</u>	<u>32,800,126</u>	<u>12,446,769</u>

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

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 129,137	\$ 52,080
Available-for-sale securities	16,232	17,014
Prepaid expenses and other current assets	3,062	1,499
Restricted cash	-	65
Total current assets	<u>148,431</u>	<u>70,658</u>
Property and equipment, net	7,935	2,429
Other non-current assets	209	-
Restricted cash	237	-
Deferred offering costs	-	3,106
Total assets	<u>\$ 156,812</u>	<u>\$ 76,193</u>
Liabilities, Preferred Units and Stockholders' / Members' Equity / (Deficit)		
Current liabilities:		
Accounts payable	\$ 4,804	\$ 5,066
Accrued expenses and other current liabilities	5,833	6,205
Total current liabilities	<u>10,637</u>	<u>11,271</u>
Other non-current liabilities	1,102	-
Total liabilities	<u>11,739</u>	<u>11,271</u>
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	322,179	-
Accumulated other comprehensive loss	(6)	(13)
Accumulated deficit	(177,135)	(124,258)
Total stockholders' / members' equity (deficit)	<u>145,073</u>	<u>(59,257)</u>
Total liabilities, preferred units and stockholders' / members' equity	<u>\$ 156,812</u>	<u>\$ 76,193</u>

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