Solid Biosciences Reports First Quarter 2019 Financial Results and Provides Business Update

May 13, 2019

– Dosing of SGT-001 initiated in second cohort of patients in the IGNITE DMD clinical trial at 2E14 vg/kg –

– Clinical activities underway at additional study sites –

CAMBRIDGE, Mass., May 13, 2019 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) today reported financial results for the first quarter ended March 31, 2019 and provided a business update.

“We have continued to advance our programs for Duchenne muscular dystrophy over the last few months, particularly our lead microdystrophin gene therapy candidate, SGT-001. Building on our findings from the preliminary Phase I/II clinical data that we announced in February, we initiated dosing at 2E14 vg/kg in the second cohort of patients,” said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. “Now, we are working to progress as quickly as possible, and we look forward to providing additional data later this year.”

Recent Developments

- Today, Solid announced that two patients have been randomized in the second cohort of the Phase I/II IGNITE DMD study, including one patient dosed with 2E14 vg/kg of SGT-001 and another added to the control group. Shortly after dosing, the patient in the treatment group was diagnosed with a gastrointestinal infection that was classified as a serious adverse event unrelated to study drug, as well as a transient decline in platelet count that was considered a non-serious adverse event related to study drug; these events have fully resolved. In addition, the patient experienced a transient elevation of transaminases, as well as a transient increase in bilirubin higher than two times the upper limit of normal, which was rapidly resolved with an increase in oral glucocorticoids. This was reported to the FDA as a serious adverse event related to study drug. The patient is doing well and has resumed normal activities. Solid continues to enroll patients in IGNITE DMD per the study protocol and anticipates providing additional data later this year.

- Solid recently initiated clinical trial activities for IGNITE DMD at additional sites, including the University of Massachusetts Memorial Medical Center. Screening continues at the University of Florida, the first clinical site for the study.

- In late April, the Company presented additional preclinical data for SGT-001 at the 22nd Annual Meeting of The American Society of Gene and Cell Therapy (ASGCT), which reinforce its potential to promote unique microdystrophin expression and rescue muscle function. Solid also presented new preclinical data at the meeting supporting its next generation gene therapy programs.

Financial Highlights

Research and development expenses for the first quarter of 2019 were $23.3 million, compared to $11.9 million for the prior year period. The increase was primarily attributable to research and development personnel and related facility costs, manufacturing costs, and clinical development costs for SGT-001.

General and administrative expenses for the first quarter of 2019 were $7.0 million, compared to $4.0 million for the prior year period. The increase was primarily attributable to increased personnel costs and other corporate expenses associated with being a public company.

Net loss for the first quarter of 2019 was $29.6 million, compared to $15.9 million for the first quarter of 2018.

Solid had $94.7 million in cash, cash equivalents and available-for-sale securities as of March 31, 2019, compared to $122.5 million as of December 31, 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 our expectations regarding the IGNITE DMD clinical trial, the safety or potential efficacy 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.
Condensed Consolidated Statements of Operations
(unaudited, in thousands, except share and per share data)

	Three Months Ended March 31,	
	2019	2018
Revenue	$ -	$ -
Operating expenses:		
Research and development	23,269	11,929
General and administrative	7,033	4,044
Total operating expenses	30,302	15,973
Loss from operations	(30,302)	(15,973)
Other income (expense):		
Interest income	508	65
Other income	212	31
Total other income (expense), net	720	96
Net loss	$ (29,582)	$ (15,877)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.85)	$ (0.54)
Weighted average shares of common stock outstanding, basic and diluted	34,776,488	29,354,650

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

	March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 53,994	$ 86,366

Available-for-sale securities	40,750	36,098
Prepaid expenses and other current assets	7,043	6,175
Total current assets	101,787	128,639
Property and equipment, net	11,995	10,422
Operating lease, right-of-use assets	5,916	-
Other non-current assets	209	209
Restricted cash	327	327
Total assets	$ 120,234	$ 139,597
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 5,352	$ 3,691
Accrued expenses	7,409	8,235
Operating lease liabilities	1,278	-
Finance lease liabilities	178	173
Other current liabilities	355	382
Total current liabilities	14,572	12,481
Operating lease liabilities, excluding current portion	5,736	-
Finance lease obligations, excluding current portion	813	859
Other non-current liabilities	-	1,074
Total liabilities	21,121	14,414
Common Stock	35	35
Additional paid-in capital	327,709	324,209
Accumulated other comprehensive gain (loss)	7	(5)
Accumulated deficit	(228,638)	(199,056)
Total stockholders' equity	99,113	125,183
Total liabilities and stockholders' equity	$ 120,234	$ 139,597

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