



## Solid Biosciences Reports Fourth Quarter and Full Year 2019 Financial Results and Provides Business Update

March 12, 2020

*– Biopsy results from the third patient dosed at 2E14 vg/kg in the SGT-001 IGNITE DMD clinical trial provide further support for continued development*

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*– Solid continues to make progress to address the IGNITE DMD clinical hold and advance the next steps for the SGT-001 program –*

CAMBRIDGE, Mass., March 12, 2020 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) today reported financial results for the fourth quarter and full year ending December 31, 2019 and provided a business update. "We are working to advance our lead program, SGT-001, a gene therapy candidate for Duchenne muscular dystrophy. We are pleased that biomarker data from all three patients dosed in the 2E14 vg/kg cohort of IGNITE DMD showed SGT-001 microdystrophin protein expression and associated neuronal nitric oxide synthase (nNOS) function, providing further evidence of the therapeutic potential of SGT-001. Our priority is to address the IGNITE DMD clinical hold so we can continue to evaluate the ability of SGT-001 to help patients with Duchenne," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences.

### Recent Developments

- Today, Solid announced biomarker data from the third patient dosed in the 2E14 vg/kg dose cohort of IGNITE DMD, the company's Phase I/II clinical trial of SGT-001, including three-month biopsy data. Using immunofluorescence assays, 50%-70% of the muscle fibers were determined to express SGT-001 microdystrophin. Immunofluorescence also showed stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin. Inclusion of the dystrophin nNOS coding region in SGT-001 may result in unique activity, potentially providing important functional benefits such as diminished muscle fatigue and protection against ischemic muscle damage. Using western blot, expression was 8% of normal control samples. The levels of serum creatine kinase, a highly variable biochemical marker of muscle damage, declined from baseline.
- In January 2020, Solid announced changes to its organizational structure to create a leaner company focused on advancing SGT-001. The corporate changes implemented reduce the company's planned corporate expenses and extend the expected cash runway.
- In December 2019, Solid announced biomarker data from two patients dosed in the 2E14 vg/kg dose cohort of IGNITE DMD. The data showed expression of SGT-001 microdystrophin and nNOS function that provides evidence SGT-001 could provide therapeutic benefit for patients with Duchenne.
- In November 2019, Solid reported that the U.S. Food and Drug Administration (FDA) placed IGNITE DMD on clinical hold following a serious adverse event in the sixth patient dosed. In December 2019, the company announced that the adverse event had fully resolved, and that the patient had resumed his normal activities. For all patients dosed in IGNITE DMD, any clinical or laboratory abnormalities observed following SGT-001 administration have fully resolved.

### Financial Highlights

Research and development expenses for the fourth quarter of 2019 were \$27.1 million, compared to \$17.8 million for the fourth quarter of 2018. Research and development expenses for the year ended December 31, 2019 were \$94.7 million, compared to \$58.0 million for the year ended December 31, 2018. The increase was primarily attributable to compensation and other costs associated with additional headcount, as well as facility costs and increased expenses related to the clinical development and manufacturing activities for SGT-001.

General and administrative expenses for the fourth quarter of 2019 were \$5.3 million, compared to \$4.6 million for the fourth quarter of 2018. General and administrative expenses for the year ended December 31, 2019 were \$24.6 million, compared to \$17.7 million for the year ended December 31, 2018. The increase was primarily attributable to increased personnel costs.

Net loss for the fourth quarter of 2019 was \$31.9 million, compared to \$21.9 million for the fourth quarter of 2018. Net loss for the year ended December 31, 2019 was \$117.2 million, compared to \$74.8 million for the year ended December 31, 2018.

Solid had \$83.5 million in cash, cash equivalents and available-for-sale securities as of December 31, 2019. Solid expects that it has sufficient capital to fund its operations into 2021.

In January 2020, Solid announced a reduction in workforce of approximately one third was implemented as part of a strategic plan designed to create a leaner company focused on advancing SGT-001. In connection with that, Solid curtailed its research and development activities supporting the company's complementary disease modifying and assistive device programs.

### About SGT-001

Solid's SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy under investigation for its ability to address the underlying genetic cause of Duchenne muscular dystrophy (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 preclinical 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, 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 (Duchenne). Founded by those touched by the disease, Solid is a center of excellence for Duchenne, bringing together experts in science, technology and care to bring forward meaningful therapies that have life-changing potential. For more information, please visit [www.solidbio.com](http://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 operations 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 satisfactorily respond to requests from the FDA for further information and data regarding IGNITE DMD; successfully resolve the clinical hold with regard to IGNITE DMD; 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 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 Duchenne 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.

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Solid Biosciences Inc.  
Consolidated Statements of Operations  
(unaudited, in thousands, except share and per share data)

	Year ended December 31,		
	2019	2018	2017
Revenue	\$ -	\$ -	\$ -
Operating expenses:			
Research and development	94,737	57,965	39,905
General and administrative	24,581	17,722	14,952
Total operating expenses	119,318	75,687	54,857
Loss from operations	(119,318)	(75,687)	(54,857)
Other income (expense):			
Revaluation of preferred unit tranche right	-	-	459
Interest income	1,580	619	219
Other income	515	270	1,001
Total other income (expense), net	2,095	889	1,679
Net loss	\$ (117,223)	\$ (74,798)	\$ (53,178)

Net loss attributable to non-controlling interest	-	-	(1,060)	)
Net loss attributable to Solid Biosciences Inc.	\$ (117,223	) \$ (74,798	) \$ (52,118	)
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	\$ (117,223	) \$ (74,798	) \$ (39,317	)
Net loss per share attributable to common stockholders, basic and diluted	\$ (2.91	) \$ (2.25	) \$ (2.88	)
Weighted average shares of common stock outstanding, basic and diluted	40,289,290	33,262,597	13,649,485	

Solid Biosciences Inc.

#### Consolidated Balance Sheets

(unaudited, in thousands, except share and per share data)

	December 31,	
	2019	2018
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 76,043	\$ 86,366
Available-for-sale securities	7,481	36,098
Prepaid expenses and other current assets	2,778	6,175
Total current assets	86,302	128,639
Operating lease, right of use assets	4,988	-
Property and equipment, net	11,645	10,422
Other non-current assets	209	209
Restricted cash	327	327
Total assets	\$ 103,471	\$ 139,597
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 7,124	\$ 3,691
Accrued expenses	9,178	8,235
Operating lease liabilities	1,736	-
Finance lease liabilities	186	173
Other current liabilities	52	382
Total current liabilities	18,276	12,481
Operating lease liabilities, excluding current portion	4,414	-
Finance lease liabilities, excluding current portion	733	859
Other non-current liabilities	-	1,074
Total liabilities	23,423	14,414
Common Stock	48	35
Additional paid-in capital	396,278	324,209
Accumulated other comprehensive gain (loss)	1	(5)
Accumulated deficit	(316,279)	(199,056)
Total stockholders' equity	80,048	125,183
Total liabilities and stockholders' equity	\$ 103,471	\$ 139,597



Source: Solid Biosciences Inc.