



## Solid Biosciences Reports Third Quarter 2020 Financial Results and Provides Business Update

November 5, 2020

*–IGNITE DMD clinical trial expected to resume dosing in the first quarter of 2021–*

*–Collaboration with Ultragenyx creates opportunities to develop additional gene therapies for Duchenne muscular dystrophy–*

*– Enhanced cash position resulting from Ultragenyx collaboration and fund-raising activity expected to provide financial runway into the second half of 2021 –*

*– Conference call and webcast scheduled for 8:30 AM ET –*

CAMBRIDGE, Mass., Nov. 05, 2020 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today reported financial results for the third quarter ended September 30, 2020 and provided a business update.

"With the FDA's lifting of the clinical hold on the IGNITE DMD trial, we are well underway in completing the activities necessary to resume dosing, which we expect will occur in the first quarter of 2021," said Ilan Ganot, Chief Executive Officer, President and Co-Founder of Solid Biosciences. "This important event and establishing a strategic collaboration with Ultragenyx to develop additional gene therapies for Duchenne mark important progress toward our goal of improving the lives of patients living with Duchenne. We are also increasing production of SGT-001 using our improved manufacturing process in support of dosing additional patients in 2021. Additionally, we strengthened our balance sheet with additional capital from the Ultragenyx collaboration and our recent at-the-market, or ATM, equity financing, both of which will support our planned clinical advancement of SGT-001."

### Recent Developments

- In [October 2020](#), Solid announced that the U.S. Food and Drug Administration (FDA) lifted the clinical hold placed on the Company's IGNITE DMD Phase I/II clinical trial. Solid expects to resume dosing in the clinical trial in the first quarter of 2021.
  - Solid implemented and shared with the FDA manufacturing process changes that remove the majority of empty viral capsids. The improved process is averaging approximately 90% full capsids, allowing target dosing to be achieved with fewer viral particles.
  - Solid submitted data from a new, quantitative, *in vitro* microdystrophin expression assay that demonstrates comparability between SGT-001 manufactured by the two processes.
  - Solid is reducing the maximum weight of the next two patients dosed to 18 kg. This reduction, in conjunction with the delivery of fewer viral particles as a result of the company's manufacturing process improvements, will reduce patients' total viral load while continuing dosing at the 2E14 vg/kg dose.
  - Solid has amended the IGNITE DMD clinical protocol to include the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and an increase in prednisone dose in the first month post dosing.
  - Solid also provided the FDA with updated functional efficacy data (including 6-Minute Walk Test and North Star Ambulatory Assessment data) for all patients dosed to date in IGNITE DMD.
- In [October 2020](#), Solid and Ultragenyx Pharmaceutical (Ultragenyx) announced a strategic collaboration to develop and commercialize new gene therapies for Duchenne. The parties will collaborate to develop products that combine Solid's differentiated microdystrophin construct and Ultragenyx's HeLa producer cell line (PCL) manufacturing platform for adeno-associated virus (AAV) vectors using AAV8 variants. The goal of the collaboration is to expand the pipeline of potential gene therapies for patients living with Duchenne.
  - Ultragenyx made a \$40 million investment in Solid at a 33% premium.
  - Ultragenyx has also agreed to pay up to \$255 million in cumulative milestone payments per product upon achievement of specified milestone events, and tiered royalties on worldwide net sales. Upon achievement of proof-of-concept, Solid has the right to opt-in to co-fund collaboration programs in return for participation in a profit share or increased royalty payments.
  - Solid retains full rights to SGT-001 as well as the opportunity to establish additional partnerships around SGT-001 or the Company's proprietary and differentiated microdystrophin construct outside of AAV8 variants.
- In October 2020, Solid announced that it sold shares of its common stock pursuant to a sales agreement dated March 13, 2019, between the Company and Jefferies LLC that resulted in gross proceeds of \$23.9 million (ATM Sale).

### Financial Highlights

Research and development expenses for the third quarter of 2020 were \$16.0 million, compared to \$22.8 million for the third quarter of 2019. Research and development expenses for the first nine months of 2020 were \$49.2 million, compared to \$67.7 million for the first nine months of 2019. The decrease was primarily attributable to a reduction in personnel and facility related expenses as a result of the restructuring that occurred in January 2020, as well as lower manufacturing costs and a decrease in costs related to other product candidates as the Company focuses on advancing SGT-001.

General and administrative expenses for the third quarter of 2020 were \$5.2 million, compared to \$6.9 million for the third quarter of 2019. General and administrative expenses for the first nine months of 2020 were \$16.0 million, compared to \$19.3 million for the first nine months of 2019. The decrease was primarily attributable to decreased personnel costs and corporate expenses partially due to the restructuring that occurred in January 2020.

Net loss for the third quarter of 2020 was \$21.2 million, compared to \$29.3 million for the third quarter of 2019. Net loss for the first nine months of 2020 was \$66.9 million, compared to \$85.4 million for the first nine months of 2019.

Solid had \$24.8 million in cash and cash equivalents as of September 30, 2020. The Company expects that its cash and cash equivalents, combined with proceeds of \$40 million from the issuance and sale of shares of common stock to Ultragenyx and the net proceeds of \$23.2 million from the ATM Sale will enable Solid to fund its operating expenses into the second half of 2021.

#### **Conference Call**

Management will host a webcast and conference call to discuss Solid's third quarter 2020 financial results and business update today, November 5, 2020, at 8:30 AM ET.

A live webcast of the call will be available on the Company's website at [www.solidbio.com](http://www.solidbio.com) under the "News & Events" tab in the Investor Relations section, or by [clicking here](#). Participants may also access the call, by dialing 866-763-0341 for domestic callers or 703-871-3818 for international callers.

The archived webcast will be available for in the "News and Events" section of the [Company's website](#).

#### **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 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, 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 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. 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 the timing and ability of the Company to resume dosing and move the IGNITE DMD clinical trial forward, the safety or potential efficacy of SGT-001, the sufficiency of the Company's cash and cash equivalents to fund its operations, potential milestone payments or royalty payments in connection with the Ultragenyx collaboration 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 resume and/or 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 patients in IGNITE DMD; 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 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 DMD/Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, 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.

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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,	
	2020	2019	2020	2019
Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	16,045	22,792	49,158	67,671
General and administrative	5,181	6,925	15,957	19,317
Restructuring charges	-	-	1,944	-
Total operating expenses	21,226	29,717	67,059	86,988
Loss from operations	(21,226)	) (29,717)	) (67,059)	) (86,988)
Other income (expense):				
Interest (expense) income	(20)	) 406	131	1,281
Other income	-	56	1	345
Total other income (expense), net	(20)	) 462	132	1,626
Net loss	\$(21,246)	) \$(29,255)	) \$(66,927)	) \$(85,362)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.44)	) \$ (0.67)	) \$ (1.39)	) \$ (2.26)
Weighted average shares of common stock outstanding, basic and diluted	48,295,468	43,467,618	48,172,686	37,727,640

Solid Biosciences Inc.

Condensed Consolidated Balance Sheets

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

	September 30, 2020	December 31, 2019
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 24,797	\$ 76,043
Available-for-sale securities	-	7,481
Prepaid expenses and other current assets	2,365	2,778
Total current assets	27,162	86,302
Property and equipment, net	8,869	11,645
Operating lease, right-of-use assets	3,951	4,988
Other non-current assets	209	209
Restricted cash	327	327
Total assets	\$ 40,518	\$ 103,471
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 4,083	\$ 7,124
Accrued expenses	8,550	9,178
Operating lease liabilities	1,930	1,736
Finance lease liabilities	202	186

Other current liabilities	-	52
Total current liabilities	14,765	18,276
Operating lease liabilities, excluding current portion	2,943	4,414
Finance lease liabilities, excluding current portion	579	733
Total liabilities	18,287	23,423
Common Stock	48	48
Additional paid-in capital	405,389	396,278
Accumulated other comprehensive income	-	1
Accumulated deficit	(383,206)	(316,279)
Total stockholders' equity	22,231	80,048
Total liabilities and stockholders' equity	\$ 40,518	\$ 103,471



Source: Solid Biosciences Inc.