Solid Biosciences Announces FDA Lifts Clinical Hold on IGNITE DMD Clinical Trial

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– Modifications to IGNITE DMD trial protocol and improvements to manufacturing processes enable continued program development –

CAMBRIDGE, Mass., Oct. 01, 2020 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold placed on the Company's IGNITE DMD Phase I/II clinical trial. As announced in July 2020, the FDA had requested further manufacturing information, updated safety and efficacy data for all patients dosed, and provided direction on total viral load to be administered per patient. Based on the Company's response to these requests, the FDA acknowledged that the Company satisfactorily addressed all clinical hold questions.

"We are pleased that our team was able to address the FDA's clinical hold questions, allowing us to restart the trial," said Carl Morris, PhD, Chief Scientific Officer at Solid Biosciences. "We are working diligently to complete all activities necessary to resume dosing, which we expect to occur in the first quarter of 2021."

As part of its commitment to continuously improve its manufacturing processes, Solid implemented and shared with the FDA manufacturing process changes that remove the majority of empty viral capsids, allowing target dosing to be achieved with fewer viral particles. This reduction in the total amount of virus delivered to each patient is intended to support safe dosing of SGT-001 for the duration of the IGNITE DMD trial. In its July 2020 communication, the FDA requested additional information regarding the comparability between SGT-001 made using Solid's prior manufacturing process and its current, improved process. In response to this request, 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 per patient, with safety outcomes from these two patients driving potential weight increase of patients dosed subsequently. 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 provided the FDA with updated safety and functional efficacy data (including 6-Minute Walk Test and North Star Ambulatory Assessment data) for all patients dosed to date in IGNITE DMD. There have been no additional drug-related adverse events up to 30 months post dosing. Additionally, to mitigate the risk of serious drug-related adverse events, Solid is amending the IGNITE DMD clinical protocol to include the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and increasing the prednisone dose in the first month post dosing.

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.

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 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 resume dosing in the IGNITE DMD trial, 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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