Solid Biosciences Announces Collaboration with Mayo Clinic to Accelerate Gene Therapy Innovation and Advance Development of Cardiac Gene Therapies

December 4, 2024

- Solid Biosciences to collaborate with Mayo Clinic's genetic cardiology disease laboratory -

- Solid to receive exclusive worldwide licenses to AAV-based Suppression-Replacement gene therapy platform and multiple next-generation cardiac gene therapy programs developed by Mayo Clinic's Windland Smith Rice Sudden Death Genomics Lab -

CHARLESTOWN, Mass., Dec. 04, 2024 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB) (the "Company" or "Solid"), a life sciences company developing precision genetic medicines for neuromuscular and cardiac diseases, today announced a collaboration with Mayo Clinic seeking to advance a cutting-edge AAV gene therapy platform for the development of next-generation therapies to treat sudden cardiac death-predisposing genetic cardiomyopathies and channelopathies with high unmet medical need and significant patient populations.

Under the terms of the agreement, Mayo Clinic's Windland Smith Rice Sudden Death Genomics Laboratory will provide Solid with an exclusive license to their Suppression and Replacement ("Sup-Rep") gene therapy platform. The collaboration will also provide Solid with an exclusive license to develop and commercialize six cardiac gene therapy programs developed using Mayo Clinic's "Sup-Rep" platform. These programs will use Solid's next-generation AAV capsids, including AAV-SLB101, and advanced manufacturing capabilities to suppress and replace genes implicated in potentially life-threatening genetic heart diseases. Mayo Clinic will be responsible for research and development efforts for each cardiac gene therapy candidate up to IND-enabling studies, at which point, Solid will have the option to pursue continued development and commercialization of each licensed program.

"Mayo Clinic's suppression-replacement gene therapy platform has the potential for a novel way of treating patients at risk for sudden cardiac death from their genetic heart disease," said Michael J. Ackerman, M.D., Ph.D., Genetic Cardiologist and Director of Mayo Clinic's Windland Smith Rice Sudden Death Genomics Lab in Rochester, MN. "It is gratifying to have the opportunity to work toward developing new therapeutic solutions for patients to help them live and thrive despite their diagnosis."

"Genetic cardiomyopathies and channelopathies represent the next frontier for the gene therapy field, and our collaboration with Dr. Ackerman and his research team places Solid at the forefront of innovation in this space," said Bo Cumbo, President and CEO of Solid. "Beginning with our CPVT IND submission expected in the first half of 2025, followed by the development of the Sup-Rep AAV platform and multiple additional therapies targeting high-impact indications, we have laid the foundation for Solid to become the leader in cardiac precision genetic medicine. We look forward to working alongside Mayo Clinic and Dr. Ackerman to transform cardiac patient care."

Mayo Clinic has a financial interest in the Company and technology referenced in this press release. Mayo Clinic will use any revenue it receives to support its not-for-profit mission in patient care, education and research.

About Solid Biosciences
Solid Biosciences is a precision genetic medicine company focused on advancing a portfolio of gene therapy candidates including SGT-003 for the treatment of Duchenne muscular dystrophy (Duchenne), SGT-501 for the treatment of catecholaminergic polymorphic ventricular tachycardia (CPVT), SGT-601 for the treatment of TNNT2-mediated dilated cardiomyopathy, SGT-401 for the treatment of BAG3-mediated dilated cardiomyopathy, and additional assets for the treatment of fatal cardiac diseases. Solid is advancing its diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management, and care. Patient-focused and founded by those directly impacted, Solid's mandate is to improve the daily lives of patients living with these devastating diseases. 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 future expectations, plans and prospects for the company; the ability to successfully achieve and execute on our collaborations with Mayo Clinic and other partners, the company's goals, priorities and achieve key clinical milestones; the company's SGT-003 program, including expectations for additional CTA filings, site activations, expanded clinical development, accelerated production of multiple GMP batches of SGT-003, initiation and enrollment in clinical trials, dosing, availability of clinical trial data and potential accelerated approval; the company's expectations for submission of an IND for SGT-501 and to submit additional INDs by the end of 2026; the cash runway of the company and the sufficiency of the Company's cash, cash equivalents, and available-for-sale securities to fund its operations; 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 advance SGT-003, SGT-501, SGT-601, SGT-401 and other preclinical programs and capsid libraries on the timelines expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; replicate in clinical trials positive results found in preclinical studies and early-stage clinical trials of the company's product candidates; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne and other neuromuscular and cardiac treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-003, SGT-501, SGT-601, SGT-401 and other candidates, 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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