



Sio Gene Therapies Provides Update on Cash Position and Major Upcoming GM1 Gangliosidosis Program Milestones

March 15, 2021

- *Company had \$120.9 million of cash and cash equivalents as of March 12, 2021*
- *Substantial increase in liquidity driven by equity investment from Suvretta Capital and sale of Arvelle shares*
- *Company estimates it is fully funded through major upcoming milestones for GM1 gene therapy program*

NEW YORK and RESEARCH TRIANGLE PARK, N.C., March 15, 2021 (GLOBE NEWSWIRE) -- Sio Gene Therapies Inc. (NASDAQ: SIOX), a clinical-stage company focused on developing gene therapies to radically transform the lives of patients with neurodegenerative diseases, today announced that it had \$120.9 million in cash and cash equivalents as of March 12, 2021, augmented by a recent \$15 million equity investment by Suvretta Capital.

Since December 31, 2020, the Company has raised \$49.1 million, of which \$37.1 million was received in gross proceeds from public sales of its common stock (including the Suvretta Capital investment) and \$11.6 million was received from a non-dilutive transaction (the sale of the Company's shares in Arvelle Therapeutics). The Company expects to receive another milestone payment of approximately \$4.8 million by mid-2021 upon the marketing approval of cenobamate by the European Medicines Agency (EMA).

"With a strengthened cash balance, we are well positioned to fulfill our patient-oriented mission by advancing our industry-leading pipeline programs well into the future. We believe that the three products in our pipeline are the most advanced gene therapies in clinical development for their respective indications, and we look forward to progressing these programs and generating additional data to support our leadership position," said David Nassif, Chief Financial Officer of Sio Gene Therapies.

The Company estimates that its current cash position extends beyond the expected dates of major upcoming milestones for the AXO-AAV-GM1 program:

1. Updated 6-month data from the low-dose cohort of late-infantile and juvenile (Type 2) children by mid-year 2021, to include biomarker data from cerebrospinal fluid (CSF) as an indicator of central nervous system activity
2. 12-month topline data from the low-dose cohort in the second half of 2021;
3. 12-month data from the first two children dosed in the high-dose cohort in Q1 2022;
4. 24-month data from the low-dose cohort in mid-2022; and
5. In the first half of 2022, meeting with the U.S. Food and Drug Administration to discuss the registrational pathway for AXO-AAV-GM1

"Thanks to the support of our clinical collaborators at the N.I.H. and the GM1 patient community, our ongoing Phase 1/2 study is currently leading the enrollment of gene therapy trials in GM1 gangliosidosis, with seven patients already enrolled across the low and high-dose cohorts. We are grateful for this support and believe our resources now extend beyond the dates of major upcoming milestones for the AXO-AAV-GM1 program," said Gavin Corcoran, MD, Chief R&D Officer. "If the data collected at these key milestones confirms, and builds upon, the preliminary results reported in December 2020, AXO-AAV-GM1 may become the first disease-modifying gene therapy to enter pivotal studies."

About Sio Gene Therapies

Sio Gene Therapies combines cutting-edge science with bold imagination to develop genetic medicines that aim to radically improve the lives of patients. Our current pipeline of clinical-stage candidates includes the first potentially curative AAV-based gene therapies for GM1 gangliosidosis and Tay-Sachs/Sandhoff diseases, which are rare and uniformly fatal pediatric conditions caused by single gene deficiencies. We are also expanding the reach of gene therapy to highly prevalent conditions such as Parkinson's disease, which affects millions of patients globally. Led by an experienced team of gene therapy development experts, and supported by collaborations with premier academic, industry and patient advocacy organizations, Sio is focused on accelerating its candidates through clinical trials to liberate patients with debilitating diseases through the transformational power of gene therapies. For more information, visit www.sioctx.com.

Forward-Looking Statements

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "believe," "estimate," "may be" and other similar expressions are intended to identify forward-looking statements. For example, all statements Sio makes regarding costs associated with its operating activities, funding requirements and/or runway to meet its upcoming clinical milestones, and timing of its upcoming clinical milestones are forward-looking. All forward-looking statements are based on estimates and assumptions by Sio's management that, although Sio believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Sio expected. Such risks and uncertainties include, among others, the impact of the Covid-19 pandemic on our operations; the actual funds and/or runway required for our clinical and product development activities and anticipated upcoming milestones; actual costs related to our clinical and product development activities and our need to access additional capital resources prior to achieving any upcoming milestones; the initiation and conduct of

preclinical studies and clinical trials; the availability of data from clinical trials; the development of a suspension-based manufacturing process for Axo-Lenti-PD; the scaling up of manufacturing, the expectations for regulatory submissions and approvals; the continued development of our gene therapy product candidates and platforms; Sio's scientific approach and general development progress; and the availability or commercial potential of Sio's product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Sio's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on February 9, 2021, as updated by its subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Sio undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Contacts:

Media

Josephine Belluardo, Ph.D.
LifeSci Communications
(646) 751-4361
jo@lifescicomms.com
info@siogtx.com

Investors and Analysts

David Nassif
Sio Gene Therapies, Inc.
Chief Financial Officer and General Counsel
(646) 677-6770
david.nassif@siogtx.com



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