Sio Gene Therapies Announces Prioritization of Lead Gene Therapy Programs in GM1 and GM2 Gangliosidosis, Extension of Cash Runway, and CEO Transition

January 31, 2022

– Company to prioritize industry-leading clinical-stage programs, AXO-AAV-GM1 and AXO-AAV-GM2, the first potential disease-modifying therapies for GM1 gangliosidosis and Tay-Sachs/Sandhoff disease, respectively; and to terminate AXO-Lenti-PD license agreement with Oxford Biomedica for Parkinson's disease

– Portfolio prioritization allows company to focus resources and extends estimated cash runway into the second half of 2023

– David Nassif, current CFO and General Counsel of Sio, appointed interim CEO following resignation of current CEO and Board Director Dr. Pavan Cheruvu

NEW YORK and DURHAM, N.C., Jan. 31, 2022 (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 provided a corporate update announcing the prioritization of AXO-AAV-GM1 and AXO-AAV-GM2, its clinical stage AAV gene therapy programs for GM1 and GM2 gangliosidosis (Tay-Sachs/Sandhoff disease). The portfolio prioritization extends the Company's estimated cash runway into the second half of 2023, beyond multiple key clinical milestones for both gene therapy programs. With this prioritization, the company intends to terminate its licensing agreement with Oxford Biomedica for AXO-Lenti-PD, its lentiviral gene therapy program for Parkinson's disease.

In addition, the Company has appointed David Nassif, J.D., its Chief Financial Officer and General Counsel, as interim Chief Executive Officer (CEO) and a member of the Board of Directors with the resignation of its CEO, Dr. Pavan Cheruvu, M.D., who is leaving the company to pursue new opportunities. A search committee of the Sio Board will consider both internal and external candidates to identify a permanent successor CEO with the assistance of a leading executive search firm.

Mr. Nassif added, "I'm honored to take the helm of Sio as we solidify the focus of the organization on rare genetic diseases and extend our cash runway into the second half of 2023. I believe our programs in GM1 and GM2 have the ability to transform the care for patients and deserve this heightened focus. We will continue to actively consider a variety of initiatives and options to further improve our financial position in order to provide the necessary capital to continue to pursue our key priorities for the longer term. We believe that pragmatic measures to grow our business in a well-managed process are aligned with the interests of patients in need, our employees and our shareholders, and we look forward to updating our progress on these efforts in the future."

"The Board is confident that Sio is in good hands with David's deep expertise in finance, law and operations, which will ensure that the company continues operating seamlessly to successfully advance our priority programs targeting GM1 and GM2 gangliosidosis. The Board is grateful for Pavan's service to the company and wishes him all the best moving forward," said Frank Torti, Chairman of the Board.

Dr. Cheruvu added, "Serving as the CEO of Sio for the last four years has been an immense privilege. I'm proud of our efforts to transform Sio into a leader in CNS gene therapies. I'm grateful to have had the opportunity to lead the company and am optimistic that its rare disease programs will have the potential to improve the lives of many children with GM1 and GM2 gangliosidosis."

Updated Financial Position

The Company provided an update on its financial resources, including having cash and cash equivalents of approximately $82 million as of December 31, 2021, and expects its estimated cash runway to fund current operations into the second half of 2023. The Company intends to report fiscal Q3 2021 results on February 11, 2022.

Program Status and Strategic Priorities

- *AXO-AAV-GM1*
  - Ten patients across all pediatric subtypes of GM1 gangliosidosis have received AXO-AAV-GM1 gene therapy to-date. The data have demonstrated a favorable risk: benefit profile and a dose-dependent improvement in key biomarkers of disease activity (β-galactosidase enzyme activity in the serum and GM1 ganglioside activity in the CSF) across the low- and high-dose cohorts
  - Strategic Priorities:
    - Calendar 1H 2022: Present a data update from Stage 1 of the Phase 1/2 study, including a first look at Type I (early-infantile) patients treated in the low-dose cohort and longer-term data from the Type II (late-infantile to juvenile) patient cohort at future scientific conferences
    - Calendar 2022: Intend to engage with the FDA to review Stage 1 data and discuss next steps for clinical development
- *AXO-AAV-GM2*
  - Dosed first four patients in the Phase 1/2 trial investigating AXO-AAV-GM2 in Tay-Sachs and Sandhoff diseases, including one patient at the starting dose and three patients at the low dose

    - Strategic Priorities:
        - 2022: Expect continued patient identification, screening, and enrollment in the mid-dose cohort (n= ~3) of the dose-ranging trial
- *AXO-Lenti-PD*
    - After a thorough analysis of the Parkinson's landscape, the company is deprioritizing its Parkinson's disease program due to several factors, including resource requirements and development timelines to reach meaningful value inflection for the program and an increasingly challenging market and regulatory environment for Parkinson's disease
    - The Company expects the rights to the program to revert back to Oxford Biomedica by March 31, 2022.

David Nassif, J.D., has served as Sio's Chief Financial Officer and General Counsel since July 2019, and has more than 25 years of life sciences industry experience in executive financial management roles in development-stage, commercial-stage, public and private companies. Prior to joining Sio, Mr. Nassif was Executive Vice President, Chief Financial Officer and General Counsel of SteadyMed Therapeutics, a specialty pharmaceutical company, where he was instrumental in its initial public offering in 2015 and in its acquisition by United Therapeutics in August 2018. From 2011 to 2014, Mr. Nassif served as the President and Chief Financial Officer of Histogen, a regenerative medicine company, where he also oversaw manufacturing and quality systems. Previously, he was Executive Vice President, General Counsel and Chief Financial Officer of Zogenix, a CNS-focused specialty pharmaceutical company and the Executive Vice President, Chief Financial Officer, General Counsel and Head of Business Development at Amphastar Pharmaceuticals, a leading generic and specialty pharmaceutical company, where he originated three company-defining strategic transactions. Earlier in his career, Mr. Nassif held various positions with Cypros Pharmaceuticals, where he was instrumental in leading its merger with Ribogene, Inc. to form Questcor Pharmaceuticals, Inc. He holds a BS in finance and management information systems with honors from the University of Virginia and a J.D. from the University of Virginia School of Law.

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. 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.siogtx.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," "expect," "intend," "estimate," "may" 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, expected cash burn runway, expectations regarding licensing and commercial agreements, and timing and outcome of its upcoming clinical and manufacturing 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 Sio's operations; the actual funds and/or runway required for Sio's clinical and product development activities and anticipated upcoming milestones; actual costs related to Sio's clinical and product development activities and Sio's 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 occurrence of adverse safety events during our current and future trials; the scaling up of manufacturing; the outcome of interactions with regulatory agencies and 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 November 12, 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.

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