Sio Gene Therapies Announces First Patient Dosed in High-Dose Cohort of AXO-AAV-GM1 Clinical Trial in Patients with GM1 Gangliosidosis

December 2, 2020

- *On-track to report topline results from low-dose cohort by end of 2020*

NEW YORK and RESEARCH TRIANGLE PARK, N.C., Dec. 02, 2020 (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 the first patient has been dosed in the high-dose cohort of the Phase 1/2 ("Stage 1") study for Type I (infantile) and Type II (late infantile and juvenile onset) GM1 gangliosidosis.

"AXO-AAV-GM1 is the only gene therapy in the clinic targeting patients with Type I and Type II GM1 gangliosidosis, a devastating and fatal pediatric disease," said Gavin Corcoran, M.D., Chief R&D Officer of Sio. "The initiation of the high-dose cohort builds on evidence of extension of survival in naturally-occurring GM1 disease animal models and encouraging clinical data from an expanded access study conducted by a National Human Genome Research Institute (NHGRI) team led by our principal investigator, Dr. Cynthia Tifft at the National Institutes of Health's (NIH) Clinical Center. Our team and academic partners are dedicated to improving the lives of children affected by this devastating disease, and we look forward to reporting topline data from the low-dose cohort before year end."

The Phase 1/2 study (NCT03952637) is designed to evaluate the safety, tolerability, and potential efficacy of AXO-AAV-GM1 delivered intravenously in patients with Type I and Type II GM1 gangliosidosis.

- The low-dose cohort evaluated $1.5x10^{13}$ vg/kg AXO-AAV-GM1 gene therapy in a total of five Type II (juvenile) patients. Six-month follow-up data from the low-dose cohort are expected by the end of 2020.
- The high-dose cohort is evaluating a dose of $4.5x10^{13}$ vg/kg AXO-AAV-GM1 gene therapy.

AXO-AAV-GM1 has received both Orphan Drug Designation and Rare Pediatric Disease Designation and is the only gene therapy in clinical development for both Type I and Type II GM1 gangliosidosis.

GM1 gangliosidosis is a progressive and fatal pediatric lysosomal storage disorder caused by mutations in the *GLB1* gene that cause impaired production of the β-galactosidase enzyme. Currently, there are no approved treatment options for GM1 gangliosidosis. In 2019, Sio reported clinically meaningful improvements from baseline to six-month follow-up for the first GM1 Type II child dosed with low-dose AXO-AAV-GM1 gene therapy under an expanded access protocol.

About AXO-AAV-GM1

AXO-AAV-GM1 delivers a functional copy of the *GLB1* gene via an adeno-associated viral (AAV) vector, with the goal of restoring β-galactosidase enzyme activity for the treatment of GM1 gangliosidosis. The gene therapy is delivered intravenously, which has the potential to broadly transduce the central nervous system and treat peripheral manifestations of the disease as well. Preclinical studies in murine and a naturally-occurring feline model of GM1 gangliosidosis have supported AXO-AAV-GM1's ability to improve β-galactosidase enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival.

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.siogtx.com.

In 2018, Sio licensed exclusive worldwide rights from the University of Massachusetts Medical School for the development and commercialization of gene therapy programs for GM1 gangliosidosis and GM2 gangliosidosis, including Tay-Sachs and Sandhoff diseases.

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 "will," "expect," "believe," "estimate," and other similar expressions are intended to identify forward-looking statements. For example, all statements Sio makes regarding costs associated with its operating activities 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 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 November 13, 2020, 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.

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
investors@siogtx.com

Source: Sio Gene Therapies