Sio Gene Therapies Announces Granting of FDA Fast Track Designation for Investigational AXO-AAV-GM1 (AAV9-GLB1) Gene Therapy in Patients with GM1 Gangliosidosis

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NEW YORK and DURHAM, N.C., Oct. 21, 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 the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to AXO-AAV-GM1, its adeno-associated viral vector (AAV)9-based gene therapy candidate for the treatment of Type I (early infantile-onset) and Type II (late infantile-onset and juvenile-onset) GM1 gangliosidosis. The Fast Track process is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need.

“Receiving Fast Track Designation is a critical step in our mission to develop the first potential treatment for all pediatric forms of this rare, terminal disease. This designation joins both the Orphan Drug Designation and Rare Pediatric Disease Designation assigned to AXO-AAV-GM1 by the FDA, which we believe further demonstrates the potential impact of this work on the patient community,” said Pavan Cheruvu, M.D., Chief Executive Officer of Sio Gene Therapies. “Building on the recently presented data at ESGCT demonstrating normalization of key disease biomarkers in the high-dose cohort with no serious adverse events attributed to AXO-AAV-GM1, this designation will help us accelerate clinical development of this promising investigational therapy for children and families.”

The current Phase 1/2 study (NCT03952637) is designed to evaluate the safety, tolerability, and potential efficacy of AXO-AAV-GM1 gene therapy delivered intravenously in children with early infantile, or Type I, and late infantile and juvenile, or Type II, GM1 gangliosidosis. Stage 1 of the study is a dose-escalation study in which the low-dose cohort is evaluating $1.5x10^{13}$ vg/kg and the high-dose cohort is evaluating a dose of $4.5x10^{13}$ vg/kg. Stage 2 of the trial will then evaluate the efficacy and safety of the optimal dose identified in Stage 1.

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 FDA-approved treatment options for GM1 gangliosidosis.

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 achieve a broad central and peripheral biodistribution. Preclinical studies in murine and a naturally-occurring feline model of GM1 gangliosidosis have supported AXO-AAV-GM1’s ability to increase β-galactosidase enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival.

AXO-AAV-GM1 has received both Orphan Drug Designation and Rare Pediatric Disease Designation from the FDA and is the only gene therapy in clinical development for all pediatric forms of GM1 gangliosidosis.

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

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.

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 "expect," "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, 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 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 occurrence of adverse safety events during our current and future trials; the development of a suspension-based manufacturing process for AXO-Lenti-PD; 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 August 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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