Sangamo Therapeutics Announces Updated Preliminary Phase 1/2 Data Showing Tolerability and Sustained Elevated α-Gal A Enzyme Activity in Patients With Fabry Disease

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- Isaralgagene civaparvovec, or ST-920, continued to be generally well tolerated across three dose cohorts in the five treated patients
- In the first two dose cohorts, all four patients exhibited above normal α-Gal A activity, ranging from 3-fold to 15-fold above mean normal; patients in the first dose cohort have maintained elevated activity for one year and are now in the long-term follow-up study
- In the third dose cohort, the fifth patient exhibited activity within mean normal α-Gal A levels at week 2 and the sixth patient was recently dosed
- Lyso-Gb3 levels remain significantly reduced in the patient who exhibited high baseline levels of this biomarker

BRISBANE, Calif.--(BUSINESS WIRE)--Feb. 7, 2022-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced updated preliminary results from the Phase 1/2 STAAR clinical study evaluating isaralgagene civaparvovec, or ST-920, a wholly owned gene therapy product candidate for the treatment of Fabry disease. These latest data show that, as of the November 9, 2021 cutoff date, the investigational treatment continued to be well tolerated and that the four longest treated patients continued to exhibit elevated alpha-galactosidase A (α-Gal A) activity. These data are being presented at the 18th Annual WORLD*Symposium™* in a platform presentation on February 8, 2022 during the 1:30 p.m. Eastern Time session and in a poster presentation available on February 7, 2022 at 6:00 p.m. Eastern Time. These data are available on the Events & Presentations page of Sangamo's website.

"These updated preliminary results demonstrate the potential of isaralgagene civaparvovec gene therapy to address the most challenging symptoms of Fabry disease with a favorable tolerability and safety profile," said Jaya Ganesh, MD, at The Icahn School of Medicine at Mount Sinai and investigator of the Phase 1/2 study. "Now with two patients dosed in the third cohort, we are eager to see if the favorable trends exhibited by patients in the first two dose cohorts continue as we follow these patients and learn more about the emerging profile of this potential treatment."

As of the cutoff date, elevated α-Gal A activity was maintained for the four patients treated in the first two dose cohorts (0.5e13 vg/kg and 1e13 vg/kg) ranging from 3-fold to 15-fold above mean normal at last measurement. For the two patients on enzyme replacement therapy (ERT), α-Gal A activity measured at ERT trough was 15-fold above mean normal at week 52 (Cohort 1) and 10-fold above mean normal at week 25 (Cohort 2). For the two ERT pseudo-naïve patients, α-Gal A activity was 3-fold above mean normal at week 52 (Cohort 1) and 4-fold above mean normal at week 40 (Cohort 2). The two patients in the first dose cohort have now begun the long-term follow-up study. For the first patient in the third dose cohort (3e13 vg/kg), α-Gal A activity has increased into mean normal range at week 2. Withdrawal from ERT has been completed for one patient and is planned for the other patient on ERT, based on the stability of their α-Gal A activity following treatment.

As of the cutoff date, isaralgagene civaparvovec was generally well tolerated across three dose cohorts in the five treated patients. There were no treatment-related adverse events higher than Grade 1 (mild) and no treatment-related serious adverse events. No patients experienced liver enzyme elevations requiring steroid treatment.

"We are very pleased with the updated preliminary results from the Phase 1/2 STAAR study and believe that isaralgagene civaparvovec gene therapy has the potential to be a compelling treatment option for patients with Fabry disease, who currently have a burdensome standard of care that requires regular and lifelong intravenous treatment, and that in many cases doesn't adequately address the underlying disease," said Rob Schott, M.D., M.P.H, F.A.C.C, Head of Development at Sangamo. "Our focus is completing the Phase 1/2 study and preparing for Phase 3 so that we can evaluate what we hope will be an important therapy in a broader Fabry patient population."

Improvements in ability to sweat were reported in the first three treated patients. No progression of Fabry cardiomyopathy was observed in the two patients experiencing cardiomyopathy prior to treatment. The one patient with a significant elevation in plasma globotriaosylsphingosine (lyso-Gb3) pre-treatment showed a significant reduction of approximately 40% (from baseline within 10 weeks after dosing, maintained through Week 36) in this biomarker after treatment with isaralgagene civaparvovec. Patients with lower baseline levels of lyso-Gb3 maintained steady levels through the cutoff date.

The sixth patient in the Phase 1/2 STAAR study, who is the second patient in the third dose cohort (3e13 vg/kg), was recently dosed after the cutoff date. Sangamo expects to provide updated results from the STAAR study throughout 2022. Sangamo is currently planning for a Phase 3 clinical trial.

About the STAAR Study

The Phase 1/2 STAAR study is a global open-label, single-dose, dose-ranging, multicenter clinical study designed to evaluate the safety and tolerability of isaralgagene civaparvovec, or ST-920, a gene therapy product candidate in patients with Fabry disease. Isaralgagene civaparvovec requires a one-time infusion without preconditioning. The STAAR study is enrolling patients who are on ERT, are ERT pseudo-naïve (defined as having been off ERT for six or more months), or who are ERT-naïve. The age range of the five patients dosed as of the cutoff date is 22 to 48 years. The U.S. Food and Drug Administration has granted Orphan Drug designation to isaralgagene civaparvovec, which has also received Orphan Medicinal Product designation from the European Medicines Agency.

About Fabry Disease

Fabry disease is a lysosomal storage disorder caused by mutations in the galactosidase alpha gene (*GLA*), which leads to deficient alpha-galactosidase A (α-Gal A) enzyme activity, which is necessary for metabolizing globotriaosylceramide (Gb3). The buildup of Gb3 in the cells can cause serious damage to vital organs, including the kidney, heart, nerves, eyes, gut and skin. Symptoms of Fabry disease can include decreased or absent sweat production, heat intolerance, angiokeratoma (skin blemishes), vision problems, kidney disease, heart failure, gastrointestinal disturbance, mood disorders, neuropathic pain and tingling in the extremities.

About Sangamo Therapeutics

Sangamo Therapeutics is a clinical-stage biopharmaceutical company with a robust genomic medicines pipeline. Using ground-breaking science, including our proprietary zinc finger genome engineering technology and manufacturing expertise, Sangamo aims to create new genomic medicines for patients suffering from diseases for which existing treatment options are inadequate or currently don't exist. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to the therapeutic potential of isaralgagene civaparvovec (ST-920), including its potential to address the most challenging symptoms of Fabry disease and to become a compelling treatment option for patients with Fabry disease, the potential for the favorable trends exhibited by patients in the first two dose cohorts of the Phase 1/2 STAAR study to continue, Sangamo's expectation for reporting updated results from the Phase 1/2 STAAR study and the expected timing thereof, plans to discontinue patients on ERT, plans for conducting a Phase 3 clinical trial of isaralgagene civaparvovec, and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to risks and uncertainties that are difficult to predict. Sangamo's actual results may differ materially and adversely from those expressed. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the uncertain timing and unpredictable nature of clinical trials and clinical trial results, including the risks that therapeutic effects observed in preliminary clinical trial results will not be durable in patients and that final Phase 1/2 STAAR study data will not validate the safety and efficacy of isaralgagene civaparvovec; reliance on results of early clinical trials, such as the Phase 1/2 STAAR study, which results are not necessarily predictive of future clinical trial results, including the results of any Phase 3 trial of isaralgagene civaparvovec; the research and development process, including the enrollment, operation and results of clinical trials and the presentation of clinical data; the effects of the evolving COVID-19 pandemic and the impacts of the pandemic on the global business environment, healthcare systems and business and operations of Sangamo, including the initiation and operation of clinical trials; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; the manufacturing of products and product candidates; the commercialization of approved products; the potential for technological developments that obviate technologies used by Sangamo in isaralgagene civaparvovec and those risks and uncertainties described in Sangamo's filings with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K for the year ended December 31, 2020 and the most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2021. The information contained in this release is as of February 7, 2022, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.

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