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

November 4, 2021

- Preliminary data showed that isaralgagene civaparvovec, or ST-920, was generally well tolerated
- All four patients in the first two dose cohorts exhibited above normal α-Gal A activity, which was maintained for up to one year for the first patient treated
- Based on these data, Sangamo has initiated Phase 3 planning
- The fifth patient was recently dosed, in the third dose cohort
- Q3 conference call and webcast scheduled for 9:15 a.m. Eastern Time

BRISBANE, Calif.--(BUSINESS WIRE)--Nov. 4, 2021-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced 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. As of the September 17, 2021 cutoff date, results from the four patients treated in the first two dose cohorts (0.5e13 vg/kg and 1e13 vg/kg) showed that isaralgagene civaparvovec was generally well tolerated. All four patients exhibited above normal alpha-galactosidase A (α-Gal A) activity, which was maintained for up to one year for the first patient treated and through 14 weeks for the most recently treated patient. Activity of 2-fold to 15-fold above mean normal was observed at last measurement as of the cutoff date. Withdrawal from enzyme replacement therapy (ERT) has taken place for one patient and is planned for the other patient on ERT, based on the stability of their α-Gal A activity following treatment.

“There is significant unmet need in Fabry disease, a progressive and challenging condition that is currently treated with frequent burdensome infusions that do not adequately address the underlying disease,” said Rob Schott, M.D., M.P.H, F.A.C.C, Head of Development at Sangamo. “We are encouraged by these early yet promising results, particularly that the first three treated patients dosed all reported improvements in their ability to sweat, which is noteworthy as this limits a person’s capability to tolerate strenuous tasks and exercise. As we continue with this study, we hope to further understand the potential treatment effect over the longer-term and in more patients, while we initiate plans for a Phase 3 trial.”

A presentation containing additional details about the study and these results is available on the Events & Presentations page of Sangamo’s website.

As of the cutoff date, 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.

The one patient with a significant elevation in plasma lyso-Gb3 pre-treatment showed significant reductions of approximately 40% (from baseline within 10 weeks after dosing, maintained through Week 32) in this biomarker after treatment with isaralgagene civaparvovec. Patients with low baseline levels of lyso-Gb3 maintained steady levels through the cutoff date.

The fifth patient in the Phase 1/2 STAAR study, who is the first patient in the third cohort (3e13vg/kg), was dosed after the cutoff date. The sixth patient is currently in screening, also for the third dose cohort. Sangamo expects to provide updated results from the STAAR study throughout 2022 and present these results at a medical meeting.

Based on STAAR study results to date, Sangamo has initiated 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 four patients dosed as of the cutoff date is 22 to 48 years old. 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.

Conference Call
Sangamo will host a conference call today, November 4, 2021, at 9:15 a.m. Eastern Time, which will be open to the public. The call will also be webcast with live Q&A and can be accessed via a link on the Sangamo Therapeutics website in the Investors and Media section under Events and Presentations.

The conference call dial-in numbers are (877) 377-7553 for domestic callers and (678) 894-3968 for international callers. The conference ID number for the call is 5178059. Participants may access the live webcast via a link on the Sangamo Therapeutics website in the Investors and Media section under Events and Presentations. A conference call replay will be available for one week following the conference call. The conference call replay numbers for domestic and international callers are (855) 859-2056 and (404) 537-3406, respectively. The conference ID number for the replay is 5178059.

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 clinical benefit to patients with Fabry disease, Sangamo's expectation for reporting updated results from the Phase 1/2 STAAR study and the expected timing thereof, 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. These risks and uncertainties are described more fully 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 November 4, 2021, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.

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