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

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- Isaralgagene civaparvovec, or ST-920, continued to be generally well tolerated across four dose cohorts in the nine treated patients in the dose escalation phase.

- All nine patients exhibited elevated α-Gal A activity, ranging from nearly 2-fold to 30-fold of mean normal, for up to 23 months post dosing, as of the last date of measurement.

- Four patients were withdrawn from enzyme replacement therapy (ERT) and demonstrated significantly elevated levels of α-Gal A activity up to 28 weeks post withdrawal.

- Since the cutoff date, one additional patient was withdrawn from ERT.

- The Phase 1/2 STAAR study has progressed into the expansion phase, with four patients dosed, including the first female patient.

BRISBANE, Calif.--(BUSINESS WIRE)--Oct. 12, 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 July 21, 2022 cutoff date, the investigational treatment continued to be generally well tolerated, with no treatment-related serious adverse events. The nine patients in the dose escalation phase exhibited elevated alpha-galactosidase A (α-Gal A) activity, sustained for up to 23 months for the patient with the longest follow-up.

These updated data will be shared today via an oral presentation at the 29th Congress of the European Society of Gene & Cell Therapy – ESGCT 2022 – from 8:30-10:40 a.m. British Summer Time (Ref: OR21). These data are also available on Sangamo's website on the Events & Presentations page.

"I'm encouraged by these results, showing isaralgagene civaparvovec gene therapy has reassuring safety data to date, with no requirement for corticosteroid therapy," said Dr Patrick Deegan, MD, Cambridge University Hospitals NHS Foundation Trust, and investigator of the Phase 1/2 study. "ST-920 has the potential to provide an alternative to enzyme replacement therapy for patients with Fabry disease. I look forward to seeing additional data as we continue to progress this important program."

As of the cutoff date, all nine patients treated in the dose escalation phase across the four dose cohorts (0.5e13 vg/kg, 1e13 vg/kg, 3e13 vg/kg and 5e13 vg/kg), sustained elevated α-Gal A activity ranging from nearly 2-fold to 30-fold of mean normal at the last date of measurement.

Cohort 1

- *Patient 1 [began the study on ERT and was subsequently withdrawn from ERT at month 19]:* α-Gal A activity was 30.4-fold of mean normal at Month 23.
- *Patient 2 [ERT pseudo-naïve]:* α-Gal A activity was 1.9-fold of mean normal at Month 18.

Cohort 2

- *Patient 3 [ERT pseudo-naïve]:* α-Gal A activity was 3.7-fold of mean normal at Month 16.
- *Patient 4 [began the study on ERT and was subsequently withdrawn from ERT at week 24]:* α-Gal A activity was 7.9-fold of mean normal at Month 12.

Cohort 3

- *Patient 5 [began the study on ERT and was subsequently withdrawn from ERT at week 29]:* α-Gal A activity was 14.7-fold of mean normal at Week 35.
- *Patient 6 [began the study on ERT and was subsequently withdrawn from ERT at week 20]:* α-Gal A activity was 4.8-fold of mean normal at Week 26.
- *Patient 7 [on ERT]:* α-Gal A activity measured at ERT trough was 8.4-fold of mean normal at Week 12.

Cohort 4

- *Patient 8 [ERT naïve]:* α-Gal A activity was 5.4-fold of mean normal at Week 16.
- *Patient 9 [ERT naïve]:* α-Gal A activity was 9.0-fold of mean normal at Week 14.

"We continue to be excited by the promising data coming from our wholly owned Fabry program. The sustained levels of α-Gal A activity after ERT withdrawal suggest that ST-920 has the potential to provide an alternative to the current standard of care," said Nathalie Dubois-Stringfellow, Ph.D, Sangamo's Senior Vice President, Chief Development Officer. "As we prepare for a potential Phase 3 trial, we look forward to sharing additional data from the nine dose escalation patients as well as our newly initiated expansion phase."

As of the cutoff date, isaralgagene civaparvovec was generally well tolerated across four dose cohorts in the nine treated patients. All treatment-related adverse events were Grade 1 (mild), except for one instance of Grade 2 (moderate) pyrexia. No treatment-related serious adverse events were reported. No patients have been treated with steroids, either prophylactically or reactively.

The two patients with the most significant elevations in plasma globotriaosylsphingosine (lyso-Gb3) showed a 40-55% reduction from baseline following treatment as of the cutoff date. Several patients experienced some increases in plasma lyso-Gb3 levels after ERT withdrawal. In these patients α-Gal A activity remained elevated, and no patients have resumed ERT.

Since the cutoff date, the fifth and final patient in the dose escalation phase who started the study on ERT has been withdrawn from ERT. The Phase 1/2 STAAR study has transitioned into the expansion phase, with the first four expansion patients dosed, including the first female patient. Sangamo is currently planning for a potential Phase 3 clinical trial.

A Current Report on Form 8-K summarizing the updated preliminary results from the Phase 1/2 STAAR clinical study in more detail will be filed by Sangamo, and this press release is subject to the further detail provided in the Form 8-K.

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 nine patients dosed as of the cutoff date is 22 to 51 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. To learn more, visit www.sangamo.com and connect with us on LinkedIn and Twitter.

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 provide an alternative to enzyme replacement therapy for patients with, and an alternative to the current standard of care for, Fabry disease, Sangamo's expectation for reporting updated results from the Phase 1/2 STAAR study on the nine dose escalation patients and the new dose expansion cohort, the continued progress of the Phase 1/2 STAAR study, 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 potential safety and efficacy of isaralgagene civaparvovec and that the patients withdrawn from ERT will remain off ERT; 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; Sangamo's lack of resources to fully develop, obtain regulatory approval for and commercialize its product candidates; 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, 2021, as supplemented by Sangamo's Quarterly Report on Form 10-Q for the quarter ended June 30, 2022. The information contained in this release is as of October 12, 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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