Sangamo Therapeutics Announces Updated Phase 1/2 STAAR Study Data in Fabry Disease Showing Sustained Benefit, Improvements in Kidney Function and Favorable Safety Profile

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Sustained benefit demonstrated with elevated expression of alpha-galactosidase A (α-Gal A) activity maintained for nearly four years for the longest treated patient as of the data cutoff date

Positive mean estimated glomerular filtration rate (eGFR) slope observed in the 23 patients who had reached at least one-year follow-up, indicating notable improvements in renal function

All 18 patients who began study on enzyme replacement therapy (ERT) have been withdrawn from, and remain off, ERT

Data to support Accelerated Approval pathway expected in first half of 2025, with potential Biologics License Application (BLA) submission to the U.S. Food and Drug Administration (FDA) anticipated in second half of 2025

Sangamo continues to advance business development discussions for a potential ST-920 collaboration

RICHMOND, Calif.--(BUSINESS WIRE)--Feb. 6, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced updated data from the Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, a wholly owned gene therapy product candidate for the treatment of Fabry disease. Updated data continue to support the potential of isaralgagene civaparvovec as a one-time, durable treatment option for Fabry disease that can improve patient outcomes.

These data will be presented at the 21st Annual WORLD*Symposium*™ in San Diego, CA on February 6, 2025, in an oral presentation in the Clinical Applications session from 9:30-10:30am P.T. and a poster presentation from 3:30-5:30pm P.T. (Poster Ref: 146). These data will also be available on Sangamo's website on the Presentations page.

"These updated data from the Phase 1/2 STAAR study are highly encouraging, particularly the positive mean eGFR slope observed in patients with at least one year of follow-up, indicating improvements in renal function, an important predicter of morbidity and mortality in Fabry disease. Additionally, these data show the strong safety and sustained benefit profiles of ST-920, as well as its ability to improve key quality of life measures," said Professor Derralynn Hughes, MA Dphil FRCP FRCPath, Royal Free London NHS Foundation Trust and investigator of the Phase 1/2 STAAR study. "These data support the potential of ST-920 to be a single-dose, durable treatment option for people living with Fabry disease."

"Following our alignment with the FDA on an Accelerated Approval Pathway for ST-920, we are thrilled with how the data are progressing, particularly the positive one-year mean eGFR slope data that will serve as the primary efficacy endpoint for our regulatory submission," said Nathalie Dubois-Stringfellow, Ph. D, Chief Development Officer at Sangamo. "We look forward to building upon the STAAR study's positive results as we advance our interactions with the FDA ahead of the potential BLA submission in the second half of 2025 and we also continue to engage with the European Medicines Agency."

Updated Phase 1/2 STAAR Study Results (as of the September 12, 2024 cut-off date)

Safety (all dosed patients):

- Isaralgagene civaparvovec continued to be generally well-tolerated, with the majority of adverse events being grade 1-2 in nature.
- No liver function test (LFT) elevations post-dosing requiring steroids occurred. No adverse events led to study discontinuation and there were no deaths.

Efficacy (all dosed patients):

- Elevated expression of α-Gal A activity maintained for up to 47 months for the longest treated patient, and up to 27 months for the longest treated patient receiving the highest dose ($2.63x10^{13}$ vg/kg).
- All 18 patients who began the study on ERT have been withdrawn from ERT and all remain off ERT as of today. Plasma lyso-Gb3 levels in these patients remained stable following ERT withdrawal for up to 33 months for the longest treated patient.
- Of the 10 patients who had measurable titers of total antibodies (Ab) or neutralizing antibodies (Nab) against α-Gal A associated with ERT at baseline, total Ab or NAb titers decreased markedly in nine patients and became undetectable in seven following ST-920 treatment.

Efficacy (23 dosed patients followed for at least 12 months):

- A positive mean annualized eGFR slope of 3.061 mL/min/1.73m^2/year (95% confidence interval: 0.863, 5.258) was observed, indicating notable improvements in kidney function.
- Improvements in disease severity were reported in the Fabry Outcome Survey adaptation of the Mainz Severity Score Index (FOS-MSSI) age-adjusted score, with 15 patients showing improvements in their total MSSI score and seven

patients improving their FOS-MSSI disease category.

- Significant improvements continued to be observed in the short form-36 (SF-36) quality of life (QoL) scores reported, with a mean change in General Health score of 10.6 (p=0.0020). For context, a three- to five-point change on any SF-36 score is considered a minimally clinically important difference.
- Significant improvements in physical component, bodily pain, physical, vitality, social function, and emotional SF-36 scores were also observed.
- Statistically significant improvements continued to be seen in the gastrointestinal symptom rating scale (GSRS) compared to baseline.
- Collectively, Sangamo believes these data continue to support the potential for isaralgagene civaparvovec as a one-time, durable treatment for Fabry disease that can improve patient outcomes.

Enrollment and dosing are complete in the Phase 1/2 STAAR study. In October 2024, Sangamo announced that the FDA had provided a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec using data from ongoing Phase 1/2 STAAR study, avoiding the requirement for an additional registrational study and accelerating estimated time to potential approval by approximately three years. The FDA agreed in a Type B interaction that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using eGFR slope at 52 weeks across all patients as an intermediate clinical endpoint.

The 52-week eGFR slope data from all enrolled patients in the Phase 1/2 STAAR study will be available in the first half of 2025. A potential BLA submission is anticipated in the second half of 2025. Sangamo continues to advance business development discussions regarding a potential ST-920 collaboration agreement.

A Current Report on Form 8-K summarizing the updated preliminary results from the Phase 1/2 STAAR study in more detail will be filed by Sangamo, and this press release is subject to the further detail provided in that 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 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 enrolled 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 FDA has granted Orphan Drug, Fast Track and RMAT designations to isaralgagene civaparvovec, which has also received Orphan Medicinal Product designation and PRIME eligibility from the European Medicines Agency and Innovative Licensing and Access Pathway from U.K. Medicines and Healthcare products Regulatory 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 genomic medicine company dedicated to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases who do not have adequate or any treatment options. Sangamo believes that its zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and that its capsid discovery platform can expand delivery beyond currently available intrathecal delivery capsids, including the central nervous system. Sangamo's pipeline also includes multiple partnered programs and programs with opportunities for partnership and investment. To learn more, visit www.sangamo.com and connect with us on LinkedIn and X.

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 safety and efficacy and therapeutic potential of isaralgagene civaparvovec, including the potential for it to be a one-time, durable treatment option for Fabry disease that can improve patient outcomes; the presentation of clinical data from the Phase 1/2 STAAR study; the potential for isaralgagene civaparvovec to qualify for the FDA's Accelerated Approval program, including the adequacy of data generated in the Phase 1/2 STAAR study to support any such approval; expectations concerning the availability of additional data to support a potential BLA submission for isaralgagene civaparvovec, and the timing of such submission; the potential to accelerate the expected timeline to approval of isaralgagene civaparvovec; Sangamo's plans to advance discussions with the FDA and the European Medicines Agency; Sangamo's plans to seek a potential collaboration partner for ST-920; and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to Sangamo's lack of capital resources to obtain regulatory approval for and commercialize its product candidates in a timely manner or at all, including the ability to secure a collaboration partner for ST-920; the uncertain timing and unpredictable nature of clinical trial results, including the risk that the therapeutic effects observed in the latest preliminary clinical data from the Phase 1/2 STAAR study will not be durable in patients and that final clinical trial data from the study will not validate the safety and efficacy of isaralgagene civaparvovec, including that the 52-week data from the Phase 1/2 STAAR study will not support a BLA submission and/or that the 104-week data from such study will not verify the clinical benefit of isaralgagene civaparvovec or support FDA approval, and that the patients withdrawn from ERT will remain off ERT; Sangamo's need for substantial additional funding to execute its operating plan and to continue to operate as a going concern; the effects of macroeconomic factors or financial challenges on the global business environment, healthcare systems and Sangamo's business and operations; the research and development process; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; the potential for technological developments that obviate technologies used by Sangamo; Sangamo's reliance on collaborators and the potential inability to secure additional collaborations; and Sangamo's ability to achieve expected future financial performance.

There can be no assurance that Sangamo and its current or potential future partners will be able to develop commercially viable products. Actual results may differ materially from those projected in these forward-looking statements due to the risks and uncertainties described above and other risks and uncertainties that exist in the operations and business environments of Sangamo and its collaborators. These risks and uncertainties are described more fully in Sangamo's Securities and Exchange Commission, or SEC, filings and reports, including in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2023, as supplemented by its Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, each filed with the SEC, and future filings and reports that Sangamo makes from time to time with the SEC. Forward-looking statements contained in this announcement are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

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