Sangamo Therapeutics Announces Positive Topline Results From Registrational STAAR Study in Fabry Disease

June 24, 2025

STAAR study demonstrated positive mean annualized estimated glomerular filtration rate (eGFR) slope at 52-weeks across all dosed patients in the study, which U.S. Food and Drug Administration (FDA) has agreed will serve as primary basis of approval

Isaralgagene civaparvovec showed a favorable safety and tolerability profile

Sangamo intends to submit a Biologics License Application (BLA) in 2026

RICHMOND, Calif.--(BUSINESS WIRE)--Jun. 24, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced positive topline results from the registrational Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, a wholly owned investigational gene therapy for the treatment of adults with Fabry disease.

Following a single dose of isaralgagene civoparvovec, a positive mean annualized eGFR slope of 1.965 mL/min/1.73m^2/year (95% confidence interval (CI): -0.153, 4.083) at 52-weeks was observed across all 32 dosed patients in the study, which the FDA has agreed will serve as an intermediate clinical endpoint under the Accelerated Approval pathway. Furthermore, a mean annualized eGFR slope of 1.747 mL/min/1.73m^2/year (95% CI: -0.106, 3.601) was observed for the 19 patients who have achieved 104-weeks of follow-up.

As recommended by the FDA, Sangamo plans to compare the annualized mean eGFR slope of isaralgagene civaparvovec with approved treatments for Fabry disease by performing a meta-analysis of published studies. According to observational studies, estimated mean annualized eGFR slopes for other marketed treatment options range from -2.2 to -0.4 mL/min/1.73m^2/year for treatments such as Replagal (agalsidase alfa)[1], Fabrazyme (agalsidase beta)[2] and Galafold (migalastat)[3].

We believe these data support the potential for isaralgagene civaparvovec as a one-time, durable treatment for Fabry disease that can improve patient outcomes and will form the basis for an anticipated BLA submission under the Accelerated Approval pathway as early as the first quarter of 2026.

The STAAR study enrolled male and female patients who were either on enzyme replacement therapy (ERT), were ERT pseudo-naïve (defined as having been off ERT for six or more months), or were ERT-naïve. The median age of patients enrolled in the study was 42, with a median duration of follow-up of 24 months and the longest treated patient having achieved 4.5 years of follow-up.

Key secondary endpoints in the study were also positive. Elevated expression of alpha-galactosidase A (α-Gal A) activity was maintained for up to 4.5 years for the longest treated patient. 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 generally stable following ERT withdrawal. A stabilization in cardiac endpoints was also observed.

Patients demonstrated a range of other clinical benefits, including improvements in disease severity reported in the Fabry Outcome Survey adaptation of the Mainz Severity Score Index (FOS-MSSI) age-adjusted score and statistically and clinically significant improvements in the short form-36 (SF-36) quality of life scores, including role-physical +14.8 (95% CI: 7.3, 22.4, p=0.0003), vitality +9.6 (95% CI: 3.9, 15.2, p=0.0017), bodily pain +9.0 (95% CI: 2.3, 15.7, p=0.0104), social functioning +7.8 (95% CI: 2.0, 13.6, p=0.0100), general health +7.4 (95% CI: 2.0, 12.8, p=0.0091), and physical component scores +4.2 (95% CI: 1.8, 6.6, p=0.0014), at week 52 compared to baseline. Statistically significant improvements in the gastrointestinal symptoms rating scale (GSRS) compared to baseline were also observed. Furthermore, following a single administration of isaralgagene civaparvovec, additional clinical benefits were observed in some patients, such as the reduction or elimination in pain medication usage and the resumption of sweating, that has enabled these patients to perform physical tasks and exercise.

Isaralgagene civaparvovec demonstrated a favorable safety and tolerability profile in the study, without the requirement for preconditioning. The majority of adverse events were grade 1-2 in nature. The most common treatment-emergent adverse events (TEAEs) were pyrexia (60.6% of participants), COVID-19 (36.4%), headache (33.3%) and nasopharyngitis (33.3%). All TEAEs resolved in response to clinical management and there were no safety-related study discontinuations.

“Fabry disease is a debilitating and multifaceted condition, for which there is a serious unmet medical need,” said Nathalie Dubois-Stringfellow, Ph. D, Chief Development Officer at Sangamo. “We are thrilled to see these compelling topline STAAR study results, including the positive mean annualized eGFR slope at both 52 and 104 weeks, alongside notable improvements in a range of secondary endpoints. Taken together these data demonstrate the potential for a single dose of ST-920 to provide meaningful clinical benefits above current standards of care and to treat the underlying pathology of Fabry disease. We want to thank the patients and investigators who participated in this study and look forward to sharing these data with health authorities.”

Isaralgagene civaparvovec has been granted Orphan Drug, Fast Track and RMAT designations from the FDA, 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.

Analyses of the full dataset from the STAAR study are ongoing and additional data will be presented at an upcoming medical meeting. Sangamo is advancing BLA preparation activities for isaralgagene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

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.

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.

[1] Replagal (agalsidase alfa) estimated mean annualized eGFR slope: -2.2 mL/min/1.73m^2/year (95% CI: -2.8, -1.7) in male patients and -0.7 mL/min /1.73m2/year (95% CI: -1.4, 0) in female patients (Source: Feriozzi, 2012: The effectiveness of long-term agalsidase alfa therapy in the treatment of Fabry nephropathy)

[2] Fabrazyme (agalsidase beta) estimated mean annualized eGFR slope: -1.5 mL/min/1.73m^2/year (Source: Fabrazyme Package Insert: https://products.sanofi.us/fabrazyme/fabrazyme.pdf)

[3] Galafold (migalastat) estimated mean annualized eGFR slope: -0.4 mL/min/1.73m^2/year (95% CI: -2.27, 1.48) (Source: Hughes, 2016: Oral pharmacological chaperone migalastat compared with enzyme replacement therapy in Fabry disease: 18-month results from the randomized phase III ATTRACT study)

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 and treat the underlying pathology of Fabry disease; 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 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 preliminary or topline data is not indicative of final results, that the therapeutic effects observed in the latest 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.

All forward-looking statements about Sangamo's future plans and expectations, including Sangamo's development plans for its product candidates, are subject to Sangamo's ability to secure adequate additional funding. 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, 2024, as supplemented by its Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, 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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