Sangamo Therapeutics Presents Detailed Data from Registrational STAAR Study in Fabry Disease at WORLDSymposium™ 2026

February 3, 2026

Data support potential of isaralgagene civaparvovec as a one-time, well tolerated and durable Fabry disease gene therapy to provide meaningful, multi-organ clinical benefits that could fundamentally shift Fabry treatment paradigm

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 can serve as primary basis of approval

Rolling submission of the Biologics License Application (BLA) to the FDA has been initiated under the Accelerated Approval pathway

RICHMOND, Calif., Feb. 03, 2026 (GLOBE NEWSWIRE) -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced detailed data from the registrational Phase 1/2 STAAR study evaluating isaralgagene civaparvovec, or ST-920, a wholly owned gene therapy product candidate for the treatment of Fabry disease.

These data will be presented via four platform presentations and in poster presentations at the 22nd Annual WORLD*Symposium*™ taking place in San Diego, CA, February 2-6, 2026. These data will also be available on Sangamo's website on the Presentations page.

"These data demonstrate the potential for the endogenous production of α-Gal A activity following ST-920 administration to transform the Fabry treatment landscape," said Dr. Robert J. Hopkin, M.D., Cincinnati Children's Hospital Medical Center, and investigator of the STAAR study. "The stabilization in cardiac function, including stability of cardiac structure and cardiac biomarkers, is particularly encouraging, given that cardiovascular disease is the most common cause of death in Fabry disease patients."

"Fabry is a challenging, multi-organ disease with insufficient current treatment options," said Dr. John A. Bernat, M.D., PhD, University of Iowa Health Care, and investigator of the STAAR study. "These encouraging data demonstrate the potential for ST-920 to improve kidney function, a notable departure from the historical renal decline characteristic of the disease. Alongside a well-tolerated safety profile, the ability to withdraw from current enzyme replacement therapy and a range of other clinical benefits, ST-920 shows the potential as a one-time, durable treatment option for Fabry disease."

Sangamo believes that the totality of data from the registrational STAAR study demonstrates the potential of isaralgagene civaparvovec as a one-time, well-tolerated and durable gene therapy treatment option for Fabry disease to provide meaningful, multi-organ clinical benefits that could fundamentally shift the Fabry treatment paradigm.

As of the April 10, 2025 data cut-off date, 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, indicating an improvement in renal function. 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 had achieved 104-weeks of follow-up. Stable cardiac function was observed over one year, including consistent cardiac structural stability across clinical and demographic subgroups. Durability of effect was demonstrated with elevated expression of alpha-galactosidase A (α-Gal A) activity maintained for up to 4.5 years for the longest treated patient, alongside statistically significant Quality of Life improvements and other clinical benefits. Isaralgagene civaparvovec demonstrated a favorable safety and tolerability profile in the study, without the requirement for preconditioning.

"Fabry is a devasting disease resulting in significantly reduced life expectancy," said Nathalie Dubois-Stringfellow, Ph.D, Chief Development Officer at Sangamo. "These compelling data demonstrate how ST-920 can alter the underlying pathology of Fabry disease to provide meaningful clinical benefit across all types of adult Fabry disease patients. We are pleased to have initiated a rolling submission of a BLA to the FDA seeking approval of ST-920."

The Phase 1/2 STAAR study is complete, and 32 patients have successfully rolled into the long-term follow-up study. The FDA has provided a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec, agreeing that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using mean annualized eGFR slope at 52 weeks as an intermediate clinical endpoint. In December 2025, Sangamo initiated a rolling submission of a BLA to the FDA seeking approval of isaralgagene civaparvovec under an Accelerated Approval pathway.

WORLD*Symposium*™ 2026 Presentations and Poster Sessions

- Isaralgagene civaparvovec (ST-920) shows positive mean annualized eGFR slope in adults with Fabry disease: Topline results from the registrational Phase 1/2 STAAR gene therapy study and long-term follow-up study
  - **Platform Presentation**: Thursday, February 5 – Clinical Applications session, 8:30-9:30am PT
  - **Poster Presentation**: Thursday, February 5, 3:30-5:30pm PT – Poster number 36

- Isaralgagene civaparvovec (ST-920) gene therapy for adults with Fabry disease: Pharmacology and immunogenicity outcomes from the Phase 1/2 STAAR study and ongoing long-term follow-up
  - **Platform Presentation**: Friday, February 6 – Contemporary Forum, Late-breaking Science session, 8:00-9:00am PT
  - **Poster Presentation**: Thursday, February 5, 3:30-5:30pm PT – Poster number 232

- Isaralgagene civaparvovec (ST-920) shows stable cardiac function over one year in patients with Fabry disease: Results from the registrational Phase 1/2 STAAR gene therapy study
    - **Platform Presentation**: Friday, February 6 – Contemporary Forum, Late-breaking Science session, 9:00-10:00am PT
    - **Poster Presentation**: Thursday, February 5, 3:30-5:30pm PT – Poster number LB-53

- A combined fertility, embryofetal development, AAV integration and germline transmission risk study in mice with isaralgagene civaparvovec (ST-920) for Fabry disease
    - **Platform Presentation**: Friday, February 6 – Rapid Fire Competition session, 1:30-1:40pm PT
    - **Poster Presentation**: Wednesday, February 4, 3:30-5:30pm PT – Poster number 252

A Current Report on Form 8-K summarizing the updated 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 was 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 were on ERT, were ERT pseudo-naïve (defined as having been off ERT for six or more months), or who were 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 in 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, well-tolerated and 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; the potential to accelerate the expected timeline to approval of isaralgagene civaparvovec; 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; the uncertain timing and unpredictable nature of clinical trial results, including the risk that the therapeutic effects observed in 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; risks related to Sangamo's reliance on third parties, including collaborators, in the development and commercialization process; 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, including as a result of the ongoing overseas conflicts, tariffs, geopolitical instability, inflation and fluctuations in interest rates, on the global business environment, healthcare systems and business and operations of Sangamo and its collaborators; the research and development process; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; reliance on results of early clinical trials, which results are not necessarily predictive of future clinical trial results, including the results of any registrational trial of Sangamo's product candidates; 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, 2024, as supplemented by its Quarterly Report on Form 10-Q for the quarter ended September 30, 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.

Contacts
Investor Relations and Media Inquiries
Louise Wilkie
ir@sangamo.com
media@sangamo.com

Source: Sangamo Therapeutics, Inc.