Sangamo Therapeutics Initiates Rolling Submission of BLA to U.S. FDA for ST-920 in Fabry Disease

December 18, 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 an endpoint to support accelerated approval pathway

Isaralgagene civaparvovec continues to show favorable safety and tolerability profile

Sangamo expects to complete Biological License Application (BLA) submission under accelerated approval pathway in second quarter of 2026

RICHMOND, Calif., Dec. 18, 2025 (GLOBE NEWSWIRE) -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, has initiated a rolling submission of a BLA to the FDA seeking accelerated approval of isaralgagene civaparvovec, or ST-920, a wholly owned investigational gene therapy for the treatment of adults with Fabry disease.

Rolling submission allows for completed modules of the BLA to be submitted and reviewed by the FDA on an ongoing basis rather than waiting for the entire BLA to be submitted at once.

"The initiation of our BLA submission marks an important milestone for Sangamo and for Fabry patients in need," said Nathalie Dubois-Stringfellow, Ph.D., Sangamo's Chief Development Officer. "The compelling data from our STAAR study shows the potential of ST-920 to provide safe and long-lasting clinical benefits to a wide range of Fabry disease patients. We look forward to working with the FDA as we continue to advance the regulatory process."

The totality of data from the registrational STAAR study demonstrated the potential for isaralgagene civaparvovec as a one-time, durable treatment of the underlying pathology of Fabry disease to provide meaningful, multi-organ, clinical benefits above current standards of care. Furthermore, the STAAR study demonstrated a positive mean annualized eGFR slope at 52-weeks across all dosed patients in the study, which the FDA has agreed will serve as endpoint to support accelerated approval.

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.

Sangamo expects to complete submission of the BLA to the FDA under the accelerated approval pathway in the second quarter of 2026.

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 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, durable treatment option for Fabry disease to provide meaningful, multi-organ clinical benefits above current standards of care; 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; 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 commercialization 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 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
Louise Wilkie
ir@sangamo.com

Media Inquiries
Melinda Hutcheon
media@sangamo.com

Source: Sangamo Therapeutics, Inc.