Sangamo Therapeutics Presents Detailed Data from Registrational STAAR Study in Fabry Disease at International Congress of Inborn Errors of Metabolism 2025

September 4, 2025

Totality of data supports 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

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 under the Accelerated Approval pathway

RICHMOND, Calif., Sept. 04, 2025 (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 investigational gene therapy for the treatment of adults with Fabry disease.

These data were presented at the International Congress of Inborn Errors of Metabolism 2025 (ICIEM2025) in Kyoto, Japan, on September 4, 2025, in a poster presentation (Poster Ref: P-662). These data are also available on Sangamo's website on the Presentations page.

"These STAAR study data demonstrate the potential for ST-920 to provide meaningful clinical benefits to Fabry disease patients," said Dr John Bernat, M.D., Ph.D., University of Iowa and investigator of the Phase 1/2 STAAR study. "The positive mean eGFR slope at both one and two years, which compares favorably to approved Fabry treatments, alongside stable cardiac function, are exciting developments, particularly given the decline in renal and cardiac function traditionally seen with Fabry patients. The ability for patients to discontinue the use of burdensome enzyme replacement therapies further supports the potential of ST-920 as a single-dose, durable treatment option for people living with Fabry disease."

"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 excited by the potential of ST-920 to provide long-lasting clinical benefits to a wide range of Fabry disease patients. Improvements in renal function and stable cardiac function, alongside quality of life and additional clinical benefits, show the ability of ST-920 to address the underlying pathology of Fabry disease and provide a potentially transformative treatment for Fabry disease patients. We look forward to sharing these data with health authorities."

Updated Phase 1/2 STAAR Study Results (as of April 10, 2025 cut-off date)

Efficacy (32 dosed patients followed at least 12 months)

- 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. This compares favorably to a meta-analysis of publications of approved Fabry treatments (Fabrazyme, Replagal and Galafold).
- Furthermore, a mean annualized eGFR slope at Week 104 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.
- Supportive mean annualized eGFR slopes were also observed across a variety of patient subgroups, including gender, baseline ERT status, Fabry disease type and baseline eGFR, showing consistency in effect across Fabry patients in the study.
- Stable cardiac function was observed, including left ventricular mass (LVM), left ventricular mass index (LVMI), left ventricular myocardial global longitudinal strain (GLS), T1 and T2 mapping, end-diastolic and end-systolic volumes that remained stable over at least one year.
- 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.
- All 18 patients who began the study on Enzyme Replacement Therapy (ERT) had been withdrawn from ERT and remained off ERT as of the data cutoff date[1]. Plasma lyso-Gb3 levels in these patients remained generally stable following ERT withdrawal.
- Of the 10 patients who had measurable titers of total antibodies (TAb) or neutralizing antibodies (Nab) against α-Gal A associated with ERT at baseline, TAb or NAb titers decreased markedly in nine patients and became undetectable in eight following treatment.
- Improvements in disease severity were reported in the Fabry Outcome Survey adaptation of the Mainz Severity Score Index (FOS-MSSI) age-adjusted score, with 22 patients showing improvements in their total MSSI score at 12 months and nine patients improving their FOS-MSSI disease category at the last assessment.
- Statistically and clinically significant improvements in the short form-36 (SF-36) quality of life scores were observed 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 were seen in the gastrointestinal symptom rating scale (GSRS) compared to baseline.

Safety (all dosed patients):

- 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 (30.3%), and nasopharyngitis (33.3%).
- All TEAEs resolved in response to clinical management and there were no safety-related study discontinuations and no deaths.

Sangamo believes these data support the potential for isaralgagene civaparvovec as a one-time, durable treatment of the underlying pathology of Fabry disease, to provide meaningful clinical benefits above current standards of care 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 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.

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 is advancing BLA preparation activities for isaralgagene civaparvovec, while continuing to engage in business development negotiations for a potential Fabry commercialization agreement.

A Current Report on Form 8-K summarizing the Phase 1/2 STAAR study data 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.

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. Moreover, Sangamo's SIFTER capsid discovery platform is advancing delivery to the central nervous system in preclinical studies. Sangamo is also progressing next generation genome editing through its modular integrase (MINT) platform. Sangamo's pipeline 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 Twitter/X.

[1] *Since the data cutoff date, a physician has decided to resume ERT for one of their treated patients who had withdrawn from ERT. This patient, who received ST-920 more than two and a half years ago, maintained supraphysiological levels of a-Gal A activity, and their lyso-Gb3 levels were generally stable as of the data cutoff date.*

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 and commercial potential of isaralgagene civaparvovec; 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 potential BLA submission for isaralgagene civaparvovec, and the timing of such submission; Sangamo's plans to seek a commercialization 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 and need for substantial additional funding to execute its operating plan and to continue to operate as a going concern, including the risk that Sangamo will be unable to obtain substantial additional funding on acceptable terms or at all or collaboration partners necessary to advance its preclinical and clinical programs, in particular for its Fabry disease program and to otherwise operate as a going concern, in which case Sangamo may be required to cease operations entirely, liquidate all or a portion of its assets and/or seek protection under the U.S. Bankruptcy Code; the uncertain timing and unpredictable nature of clinical trial results, including the risk 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 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; 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 Sangamo's potential inability to secure additional collaborations; 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 June 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.

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Source: Sangamo Therapeutics, Inc.