Sangamo Therapeutics Announces Important Derisking Milestones in Pathway to Anticipated BLA Submission for ST-920 in Fabry Disease

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- *All dosed patients have passed one-year milestone required by U.S. Food and Drug Administration (FDA) for Accelerated Approval regulatory pathway for ST-920.*
- *According to preliminary analysis, mean estimated glomerular filtration rate (eGFR) slope at 52-weeks continued to remain positive.*
- *Productive Type B Chemistry, Manufacturing and Controls (CMC) meeting with FDA provided clear CMC pathway to planned Biologics License Application (BLA) submission.*
- *Pivotal data readout expected by end of second quarter of 2025.*

RICHMOND, Calif.--(BUSINESS WIRE)--May 6, 2025-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced important derisking events in the pathway to a planned BLA submission for isaralgagene civaparvovec, or ST-920, its wholly owned gene therapy product candidate for the treatment of Fabry disease.

All dosed patients in the Phase 1/2 STAAR study evaluating isaralgagene civaparvovec have now completed at least 52-weeks of follow-up, a key milestone required by the FDA for an Accelerated Approval regulatory pathway for ST-920. Preliminary analysis of clinical data collected as of this 52-week milestone date across all 32 dosed patients indicates that the mean eGFR slope continued to remain positive, following the last clinical update at the WORLD*Symposium* in February 2025, with a data cutoff date of September 12, 2024. The product candidate continues to be well tolerated. A pivotal data readout is expected by the end of the second quarter of 2025.

Furthermore, in April 2025, Sangamo held a productive Type B meeting with the FDA, providing Sangamo with a clear CMC pathway to a planned BLA submission in the first quarter of 2026, including clarity on plans for process validation, path to commercial specifications and the commercial launch manufacturing site. This BLA submission timeline would facilitate a potential approval and commercial launch as early as the second half of 2026.

“Following last year’s alignment with the FDA on an Accelerated Approval regulatory pathway for ST-920, we are excited to have now gathered the one-year mean eGFR slope data that will serve as the primary efficacy endpoint for our planned BLA submission,” said Nathalie Dubois-Stringfellow, Ph. D., Chief Development Officer at Sangamo. “Coupled with our recent productive FDA Type B meeting, we have a clear regulatory pathway to a potential approval decision for ST-920 and we continue to advance BLA preparation activities.”

Discussions with the European Medicines Agency (EMA) on the proposed pathway to potential approval for isaralgagene civaparvovec in Europe are ongoing. In addition, Sangamo continues 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 the safety and tolerability of 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 male and female 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 EMA 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; 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 serve as the primary efficacy endpoint for, and otherwise support, any such approval; expectations concerning the timing of the pivotal data readout and the

availability of additional data to support a potential BLA submission for isaralgagene civaparvovec, and the timing of such submission; expectations concerning Sangamo's regulatory pathway for isaralgagene civaparvovec, including potential regulatory approval and commercial launch of isaralgagene civaparvovec and the timing thereof; *Sangamo's plans to seek a potential collaboration partner for isaralgagene civaparvovec; expectations concerning potential approval of isaralgagene civaparvovec in other jurisdictions; 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 Sangamo's ability to secure a partnership for isaralgagene civaparvovec and its other product candidates; 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; the uncertain regulatory approval process, including the risk that Sangamo will not be able to achieve regulatory approval of isaralgagene civaparvovec on the expected timeframe or at all; risks related to the complex process of manufacturing product candidates; 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 collaborators 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 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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