Sangamo Therapeutics Announces Alignment With FDA on Accelerated Approval Pathway for ST-920 in Fabry Disease With BLA Submission Expected in 2025

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- U.S. Food and Drug Administration (FDA) provides a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec using data from ongoing Phase 1/2 STAAR study, avoiding requirement for additional registrational study and accelerating estimated time to potential approval by approximately three years.

- FDA confirms that estimated glomerular filtration rate (eGFR) slope data at one year across all Phase 1/2 patients can serve as primary basis for approval under Accelerated Approval pathway.

- Data to support Accelerated Approval pathway available in first half of 2025, with a potential Biologics License Application (BLA) submission expected in the second half of 2025.

RICHMOND, Calif.--(BUSINESS WIRE)--Oct. 22, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced the outcome of a recent successful interaction with the U.S. FDA, providing a clear regulatory pathway to Accelerated Approval for isaralgagene civaparvovec, or ST-920, its wholly owned gene therapy product candidate for the treatment of Fabry disease.

The FDA has agreed in a Type B interaction that data from the ongoing Phase 1/2 STAAR study can serve as the primary basis for approval under the Accelerated Approval Program, using eGFR slope at 52 weeks across all patients as an intermediate clinical endpoint. The complete dataset to support an Accelerated Approval pathway will be available in the first half of 2025. This approach unlocks a potential BLA submission in the second half of 2025, three years ahead of previous estimates, and avoids the requirement for an additional registrational study to establish clinical efficacy.

Sangamo engaged with the FDA on alternative pathways to potential approval following analysis of clinical data from the Phase 1/2 STAAR study showing encouraging safety and efficacy data, including promising preliminary evidence of improved kidney function. Renal manifestations, such as proteinuria or a decreased glomerular filtration rate, occur early in life in almost all male, and in many female, patients with Fabry disease, and can lead to end-stage renal disease and early death. In the 18 male and female patients treated with isaralgagene civaparvovec with more than one year of follow-up data, statistically significant improvements were observed in both mean and median eGFR levels, resulting in a positive annualized eGFR slope. Based on these latest data, the FDA agreed that eGFR slope at 52 weeks can serve as an intermediate clinical endpoint to support a potential Accelerated Approval. The FDA also advised that eGFR slope at 104 weeks may be assessed to verify clinical benefit.

“Fabry is a debilitating disease, for which there is a serious unmet medical need,” said Sandy Macrae, Chief Executive Officer of Sangamo. “I strongly believe in the potential for ST-920 to alleviate many manifestations of Fabry disease and am delighted to have a clear regulatory pathway that could bring this treatment to patients significantly sooner than originally anticipated”.

Dosing was completed in the Phase 1/2 STAAR study in April 2024, with a total of 33 patients dosed. The longest treated patient recently achieved four years of follow-up. The 18th and final patient who started the study on Enzyme Replacement Therapy (ERT) was successfully withdrawn from ERT in September 2024, and all 18 patients remain off ERT as of today. The 52-week eGFR slope data from all enrolled patients in the Phase 1/2 STAAR study will be available in the first half of 2025. A potential BLA submission is anticipated in the second half of 2025.

Sangamo has begun to execute BLA readiness activities for isaralgagene civaparvovec, while continuing to advance ongoing business development discussions with potential collaboration partners.

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 our 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 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 and bring isaralgagene civaparvovec to patients sooner than previously expected; *the anticipated advancement of isaralgagene civaparvovec to registration, including Sangamo's plans to seek a potential collaboration partner; 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 our lack of capital resources to obtain regulatory approval for and commercialize our product candidates in a timely manner or at all, including our ability to secure a partnership; 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; our need for substantial additional funding to execute our 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 our 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; our reliance on collaborators and our potential inability to secure additional collaborations; and our ability to achieve expected future financial performance.*

There can be no assurance that we and our 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 our collaborators. These risks and uncertainties are described more fully in our Securities and Exchange Commission, or SEC, filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2023, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, 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 we undertake no duty to update such information except as required under applicable law.

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