Sangamo Therapeutics Announces U.S. FDA Alignment on Abbreviated Pathway to Potential Approval and EMA Prime Eligibility for ST-920 in Fabry Disease

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- U.S. Food and Drug Administration (FDA) advises that a single study with up to 25 patients, in combination with confirmatory evidence, may be acceptable pathway to Biologics License Application (BLA) submission for isaralgagene civaparvovec, which would significantly reduce anticipated complexity, cost and time to potential approval.

- European Medicines Agency (EMA) granted priority medicines (PRIME) eligibility to isaralgagene civaparvovec, which includes enhanced regulatory support and scientific guidance.

- Sangamo is actively seeking a collaboration partner to advance isaralgagene civaparvovec through potential registration and commercialization.

RICHMOND, Calif.--(BUSINESS WIRE)--Feb. 12, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced important U.S. and European regulatory updates 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 D meeting that data from a single, adequate, and well-controlled study may form the primary basis of approval of a BLA for isaralgagene civaparvovec. The proposed study would enroll up to 25 patients, both male and female, without the need for a control arm. A head-to-head comparison with Enzyme Replacement Therapy (ERT) is not part of the proposed study design deemed acceptable by the FDA. This approach enables a potentially more rapid, efficient and cost-effective pathway to BLA submission than originally anticipated.

Additionally, the EMA has granted PRIME eligibility to isaralgagene civaparvovec. PRIME is a program designed to enhance support for the development of medicines that target an unmet medical need and is intended to optimize development plans and expedite review and approval processes so that these medicines may reach patients as early as possible. Isaralgagene civaparvovec has already received Orphan Medicinal Product designation from the EMA as well as Orphan Drug, Fast Track and RMAT designations from the FDA.

“The U.S. and European regulatory support for ST-920 and the serious unmet medical need in Fabry Disease signal the important role that ST-920 could play in improving the lives of Fabry patients across the globe,” said Nathalie Dubois Stringfellow, Ph.D., Chief Development Officer of Sangamo. “We are thankful for the FDA’s support and alignment on a regulatory pathway that could potentially deliver a new treatment option for Fabry disease patients on an expedited, cost-effective timeline. Similarly, we appreciate the support from the EMA and the opportunity to advance our development plans in Europe. Fabry is a debilitating disease in need of new medicines, and we are grateful that regulatory agencies across geographies recognize this and support our proposed development plans.”

Updated Phase 1/2 STAAR study data showing sustained clinical benefit and a differentiated safety profile across 24 patients were shared at the 20th Annual WORLD*Symposium*™ in San Diego, CA on Wednesday, February 7, 2024. A total of 29 patients have been treated to date in the Phase 1/2 STAAR study. All 13 patients withdrawn from ERT remain off ERT as of February 12, 2024. Screening and enrollment are complete in the study and dosing of the remaining enrolled patients is expected in the first half of 2024. Sangamo is deferring additional investments in planning for a registrational trial until a collaboration partnership is secured.

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 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.

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’s zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and Sangamo’s capsid discovery platform is making progress toward potentially expanding 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 Twitter/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 and commercial potential of isaralgagene civaparvovec, the anticipated plans and timelines for conducting our ongoing and potential future clinical trials and presenting clinical data from our clinical trials, expectations regarding the conclusion of dosing in our Phase 1/2 STAAR study, the anticipated advancement of isaralgagene civaparvovec to late-stage development, including Sangamo's plans to seek a potential partner to proceed with potential future registrational studies of isaralgagene civaparvovec, the design of any potential future studies of isaralgagene civaparvovec, the potential impact of FDA and EMA feedback on the regulatory pathway for 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 our lack of capital resources to fully develop, obtain regulatory approval for and commercialize our product candidates, including our ability to secure a partnership required to initiate a potential registrational study of isaralgagene civaparvovec in a timely manner or at all; 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, including as a result of the ongoing overseas conflict, current or potential future bank failures, inflation and elevated interest rates, on the global business environment, healthcare systems and business and operations of Sangamo and our collaborators, including the operation of clinical trials; the research and development process, including the operation and results of clinical trials and the presentation of clinical data; the impacts of clinical trial delays, pauses and holds on clinical trial timelines and commercialization of 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, and that the patients withdrawn from ERT will remain off ERT; 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 studies of our product candidates; 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, 2022, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, 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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