Sangamo Therapeutics Receives U.S. FDA Fast Track Designation for Isaralgagene Civaparvovec for the Treatment of Fabry Disease

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BRISBANE, Calif.--(BUSINESS WIRE)--May 22, 2023-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to isaralgagene civaparvovec, or ST-920, a wholly owned gene therapy product candidate for the treatment of Fabry disease.

Fast Track designation aims to facilitate the development and expedite the review of new therapeutics that are intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. Companies granted this designation are given the opportunity for more frequent interactions with the FDA. These clinical programs may also be eligible to apply for Accelerated Approval and Priority Review if relevant criteria are met. The FDA has previously granted ST-920 Orphan Drug Designation.

"We are thrilled with the FDA's decision to grant Fast Track Designation for ST-920. Fabry is a debilitating disease with life-long impact," said Nathalie Dubois-Stringfellow, Ph.D, Sangamo's Senior Vice President, Chief Development Officer. "This decision from the FDA underscores the potential for ST-920 to address a serious unmet need and serve as a meaningful therapeutic option for patients with Fabry disease. We are highly encouraged by this promising development and look forward to our expected meeting with the FDA on Phase 3 trial design in the summer."

ST-920 is currently being evaluated in the Phase 1/2 STAAR study, with a total of 20 patients dosed to date. In February 2023, Sangamo announced promising results from the STAAR study via an oral presentation at the 19th Annual WORLD*Symposium,* showing sustained, elevated expression of alpha-galactosidase A (α-Gal A) activity in the 13 dosed patients as of the data cutoff, 78% globotriaosylceramide (Gb3) substrate clearance at 6-months and 77% reduction in urine podocyte loss in one of the first kidney biopsies, and a clinically meaningful and statistically significant increase in mean general health scores, as measured by the SF-36 General Health survey. A copy of the presentation is available in the Presentations section of the Sangamo website. Sangamo is currently preparing for a potential Phase 3 trial and plans to meet with the FDA on the proposed Phase 3 study design in the summer, with a trial start anticipated by the end of 2023, depending on regulatory interactions.

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 is enrolling 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 U.S. Food and Drug Administration has granted Orphan Drug and Fast Track designation to isaralgagene civaparvovec, which has also received Orphan Medicinal Product designation from the European Medicines 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 clinical-stage biopharmaceutical company with a robust genomic medicines pipeline. Using ground-breaking science, including our proprietary zinc finger genome engineering technology and manufacturing expertise, Sangamo aims to create new genomic medicines for patients suffering from diseases for which existing treatment options are inadequate or currently don't exist. To learn more, visit www.sangamo.com and connect with us on LinkedIn and Twitter.

Sangamo 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 ability of the Fast Track designation to facilitate the development and expedite the review of isaralgagene civaparvovec (ST-920), the ability to meet relevant criteria in order to be eligible to apply for Accelerated Approval and Priority Review, the therapeutic potential of isaralgagene civaparvovec, including its potential to improve the lives of patients without the need for ERT and to have a favorable effect on Fabry nephropathy, Sangamo's expectation for, and timelines related to, the completion of dosing in the Phase 1/2 STAAR study, plans to meet with the FDA on the proposed Phase 3 study design, the planning for and the anticipated commencement of and dosing in a potential Phase 3 trial, depending on regulatory interactions, and the anticipated timing thereof, and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to risks and uncertainties that are difficult to predict. Sangamo's actual results may differ materially and adversely from those expressed. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the uncertain timing and unpredictable nature of clinical trials and clinical trial results, including the risks that therapeutic effects observed in preliminary clinical trial results, including data from kidney biopsies, will not be durable in patients and that final Phase 1/2 STAAR study data will not validate the potential safety and efficacy of isaralgagene civaparvovec and that the patients withdrawn from ERT will remain off ERT; reliance on results of early clinical trials, such as the Phase 1/2 STAAR study, which results are not necessarily predictive of future clinical trial results, including the results of any Phase 3 trial of isaralgagene civaparvovec; the research and development process, including the enrollment, operation and results of clinical trials and the presentation of clinical data; the effects of the COVID-19 pandemic and the impacts of the pandemic on the global business environment, healthcare systems and business and operations of Sangamo, including the initiation and operation of clinical trials; the unpredictable

regulatory approval process for product candidates across multiple regulatory authorities; the manufacturing of products and product candidates; the commercialization of approved products; the potential for technological developments that obviate technologies used by Sangamo in isaralgagene civaparvovec; Sangamo's lack of resources to fully develop, obtain regulatory approval for and commercialize its product candidates; and those risks and uncertainties described in Sangamo's filings with the U.S. Securities and Exchange Commission, or the SEC, including its Annual Report on Form 10-K for the year ended December 31, 2022, as supplemented by its Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, 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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Investor Relations & Media Inquiries
Louise Wilkie
ir@sangamo.com
media@sangamo.com

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