Sangamo Therapeutics to Regain Full Rights to Hemophilia A Gene Therapy Program Following Pfizer's Decision to Cease Development of Giroctocogene Fitelparvovec

December 30, 2024

- Based on positive results from Phase 3 AFFINE trial, which met primary and secondary endpoints, Sangamo plans to explore all options to commercialize the asset, including seeking a potential new collaboration partner

RICHMOND, Calif.--(BUSINESS WIRE)--Dec. 30, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced it will regain development and commercialization rights to giroctocogene fitelparvovec, an investigational gene therapy product candidate for the treatment of adults with moderately severe to severe hemophilia A that it has co-developed with, and licensed to Pfizer Inc., following a decision by Pfizer to terminate the global collaboration and license agreement between the parties. Sangamo intends to explore all options to advance the program, including seeking a potential new collaboration partner. Pfizer has indicated to Sangamo that this termination reflects its decision not to proceed with the Biologics License Application (BLA) and Marketing Authorisation Application (MAA) submissions for, or to pursue commercialization of, giroctocogene fitelparvovec.

In July 2024, Pfizer announced positive topline results from the Phase 3 AFFINE trial evaluating giroctocogene fitelparvovec that demonstrated the trial met primary and key secondary objectives of superiority compared to prophylaxis. On December 9, 2024, Pfizer presented detailed AFFINE data in an oral presentation at the 66th ASH Annual Meeting and Exposition, with the presentation selected as part of the "Best of ASH" program, which highlights the most cutting-edge science presented during the Annual Meeting. In November 2024, Pfizer indicated to Sangamo that Pfizer was discussing these data with regulatory authorities. Pfizer previously indicated to Sangamo that anticipated BLA and MAA submissions were expected to occur in early 2025.

"Giroctocogene fitelparvovec has demonstrated the potential to be a life changing gene therapy treatment for hemophilia A patients, and following positive results from the Phase 3 AFFINE trial, we believe it is well positioned for regulatory submissions and potential commercialization," said Sandy Macrae, Chief Executive Officer of Sangamo Therapeutics. "While we were surprised and extremely disappointed by Pfizer's decision to end our collaboration so close to the anticipated BLA and MAA submissions, especially given the compelling pivotal clinical trial data, we appreciate their collaboration in leading a robust and successful clinical development program and for advancing the asset to this important stage. We are committed to exploring the optimal path forward for this important treatment, including seeking the right partner with the focus and understanding of the genomic medicine commercial environment to bring this medicine to patients. In parallel, we remain focused on advancing our wholly owned neurology genomic medicine pipeline and progressing our Fabry gene therapy program towards a potential BLA submission in the second half of 2025."

The collaboration and license agreement with Pfizer will terminate effective April 21, 2025, at which time Pfizer will be required to transition the giroctocogene fitelparvovec program back to Sangamo. All trial participants will continue to be monitored as planned during the transition period.

Sangamo believes that its recently announced partnerships with Genentech and Astellas, and advanced business development discussions for its Fabry gene therapy program, will allow it to chart a path forward for its neurology genomic medicine pipeline as it prepares to initiate expected enrollment of patients in the Phase 1/2 study of ST-503 for idiopathic small fiber neuropathy in mid-2025, and file an anticipated Clinical Trial Authorisation (CTA) submission for the prion disease program in Q4 2025, each subject to securing adequate additional funding.

About the AFFINE Trial

The Phase 3 AFFINE (NCT04370054) trial is an open-label, multicenter, single-arm trial to evaluate the efficacy and safety of a single infusion of giroctocogene fitelparvovec in adult male participants (n=75 dosed participants) with moderately severe to severe hemophilia A. Trial participants included in the assessments of the key endpoints of the primary efficacy analysis (n=50) completed a minimum six months of routine FVIII replacement prophylaxis therapy during the lead-in study (NCT03587116) providing data to compare with post giroctocogene fitelparvovec treatment.

In July 2024, Pfizer announced that the AFFINE trial achieved its primary objective of non-inferiority, as well as superiority, of total annualized bleeding rate (ABR) from Week 12 through at least 15 months of follow up post-infusion compared with routine Factor VIII (FVIII) replacement prophylaxis treatment. Following a single 3e13 vg/kg dose, giroctocogene fitelparvovec demonstrated a statistically significant reduction in mean total ABR compared to the pre-infusion period (1.24 vs 4.73; one-sided p-value=0.0040). Key secondary endpoints as defined by the trial protocol were met and also demonstrated superiority compared to prophylaxis. In the AFFINE trial, giroctocogene fitelparvovec was generally well tolerated.

Giroctocogene fitelparvovec has been developed as part of a collaboration agreement for the global development and commercialization of gene therapies for hemophilia A between Sangamo Therapeutics and Pfizer. In late 2019, Sangamo transferred the manufacturing technology and the Investigational New Drug application to Pfizer. Under the agreement, Pfizer assumed responsibility for pivotal studies, any regulatory activities, and potential global commercialization of giroctocogene fitelparvovec.

About Hemophilia A

Hemophilia is an inherited, rare bleeding disorder that causes people to bleed for longer than normal due to a deficiency of a protein required for normal blood clotting, known as clotting Factor VIII (FVIII) in hemophilia A. The severity of hemophilia is determined by the amount of the factor in the blood. The lower the amount of the factor, the more likely it is that bleeding will occur, which can lead to serious health problems.

Hemophilia A occurs in approximately 25 in every 100,000 male births worldwide. Approximately 55-75% of males with hemophilia A have a moderate to severe form of the disease. For people who live with hemophilia A, there is an increased risk of spontaneous bleeding as well as bleeding following injuries or surgery. It is a lifelong disease that requires constant monitoring and therapy.

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 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: Sangamo's plans to explore options to commercialize giroctocogene fitelparvovec and advance the giroctocogene fitelparvovec program, including through seeking a potential new collaboration partner; the potential of giroctocogene fitelparvovec to be a life changing gene therapy treatment for hemophilia A patients; Sangamo's belief that giroctocogene fitelparvovec is well positioned for regulatory submissions and potential commercialization; the effects of the termination of the collaboration and license agreement with Pfizer, including with respect to the transition of giroctocogene fitelparvovec back to Sangamo; expectations concerning Sangamo's Fabry program, including plans for a potential BLA submission and the timing thereof and expectations concerning Sangamo's ability to secure a potential partnership for such program; Sangamo's plans to initiate enrollment of patients in the Phase 1/2 study of ST-503 for idiopathic small fiber neuropathy and the anticipated timing thereof; Sangamo's plans to submit a CTA for the prion disease program and the anticipated timing thereof; Sangamo's belief that its recently announced partnerships with Genentech and Astellas, and advanced business development discussions for its Fabry gene therapy program, will allow it to chart a path forward for its neurology genomic medicine pipeline; 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 funding or partnerships or additional collaboration partners necessary to advance its preclinical and clinical programs 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 and costly research and development process; risks related to Sangamo's reliance on partners to advance its Fabry and giroctocogene fitelparvovec programs, including the risks that Sangamo may be unable to identify and engage any new potential collaboration partners for such programs on commercially reasonable terms or at all, and even if Sangamo is able to do so, such potential partners may be unable to meaningfully advance such programs, whether due to internal portfolio prioritization decisions or otherwise; the risk that Sangamo will not realize the benefits of, including receipt of potential payments under, its collaboration agreements; the effects of macroeconomic factors or financial challenges, including as a result of the ongoing overseas conflicts, current or potential future bank failures, inflation and high interest rates, on the global business environment, healthcare systems and business and operations of Sangamo and its collaborators; the uncertain timing and unpredictable nature of clinical trial results, including the risk that therapeutic effects in the Phase 3 AFFINE trial will not be durable in patients; 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; and the potential for technological developments that obviate technologies used by Sangamo. There can be no assurance that Sangamo and its 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, 2023, as supplemented by Sangamo's Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, and subsequent filings and reports that Sangamo makes from time to time with the SEC. Forward-looking statements contained in this press release 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 and Media Inquiries
Louise Wilkie
ir@sangamo.com
media@sangamo.com

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