Pfizer and Sangamo Announce Updated Phase 1/2 Results Showing Sustained Bleeding Control in Highest Dose Cohort Through Two Years Following Hemophilia A Gene Therapy

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NEW YORK & BRISBANE, Calif.--(BUSINESS WIRE)--Dec. 12, 2021-- Pfizer Inc. (NYSE: PFE) and Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today announced updated follow-up data from the Phase 1/2 Alta study of giroctocogene fitelparvovec, an investigational gene therapy for patients with moderately severe to severe hemophilia A. The Alta study data, in patients with severe hemophilia A, are being presented today at the 63rd American Society for Hematology Annual Meeting and Exposition taking place from December 11-14 virtually and in Atlanta, GA. The oral presentation slides, which include follow-up data up to 195 weeks for the longest-treated patient, are available on Sangamo's website in the Investors and Media section under Events and Presentations.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20211212005051/en/

At 104 weeks, the five patients in the highest dose 3e13 vg/kg cohort had mean factor VIII (FVIII) activity of 25.4% via chromogenic clotting assay. In this cohort, mean annualized bleeding rate (ABR) was 0.0 in the first year post-infusion and was 1.4 throughout the total duration of follow-up as of the October 1, 2021 cutoff date. All bleeding events occurred after week 69 post-infusion. Two patients experienced bleeding events necessitating treatment with exogenous FVIII. No participants in the highest dose cohort have resumed prophylaxis.

"These latest results further suggest the potential of this investigational therapy to bring transformational benefit to eligible patients living with severe hemophilia A, if confirmed in ongoing clinical trials," said Seng H. Cheng, Senior Vice President and Chief Scientific Officer, Pfizer Rare Disease.

"We continue to be encouraged by findings from the Phase 1/2 Alta study in patients with severe hemophilia A," said Rob Schott, M.D., M.P.H, F.A.C.C, Head of Development at Sangamo. "We believe these two-year results demonstrate the potential of this gene therapy candidate to minimize significant symptoms associated with hemophilia A and become an alternative to the current burden of disease management."

Giroctocogene fitelparvovec was generally well-tolerated in this Phase 1/2 study. Among the five patients in the highest dose cohort, four received corticosteroids for liver enzyme (ALT/AST) elevations. All elevations fully resolved with oral corticosteroids. As previously reported, one patient in the highest dose cohort had a treatment-related serious adverse event of hypotension (grade 3) and fever (grade 2), with symptoms of headache and tachycardia, which occurred six hours post-infusion with giroctocogene fitelparvovec and resolved approximately 12 hours post-infusion. Across all four cohorts, 26 treatment-related adverse events occurred in six patients as of the October 1, 2021 cutoff date. No other treatment-related serious adverse events were reported as of the cutoff date. Additionally, no confirmed FVIII inhibitor development occurred, and no thrombotic events were reported.

The Phase 3 AFFINE clinical trial of giroctocogene fitelparvovec in patients with hemophilia A has started and is over 50% enrolled. Following the observation of FVIII levels greater than 150% in some treated patients, Pfizer voluntarily paused screening and dosing of additional patients in the trial to implement a protocol amendment to provide clinical management guidance for elevated FVIII levels. Subsequently, on November 3, 2021, the U.S. Food and Drug Administration (FDA) informed Pfizer that this trial has been placed on clinical hold while the protocol amendment and associated documents are reviewed.

About the Alta Study

The Phase 1/2 Alta study is an open-label, dose-ranging, multicenter clinical trial designed to assess the safety and tolerability of giroctocogene fitelparvovec in patients with severe hemophilia A. The mean age of the 11 male patients assessed across four dose cohorts (9e11 vg/kg - 2 patients, 2e12 vg/kg - 2 patients, 1 e13 vg/kg - 2 patients and 3e13 vg/kg - 5 patients) is 30 years (range 18-47 years). Patients in this study will be assessed every six months until they enroll in a long-term follow-up study.

About the AFFINE study

The Phase 3 AFFINE (NCT04370054) study is an open-label, multicenter, single arm study to evaluate the efficacy and safety of a single infusion of giroctocogene fitelparvovec in more than 60 adult (ages 18-64 years) male participants with moderately severe to severe hemophilia A. Eligible study participants will have completed at least six months of routine FVIII prophylaxis therapy during the lead-in Phase 3 study (NCT03587116) in order to collect pretreatment data for efficacy and selected safety parameters.

The primary endpoint is impact on annualized bleeding rate (ABR) through 12 months following treatment with giroctocogene fitelparvovec. This will be compared to ABR on prior FVIII prophylaxis replacement therapy. The secondary endpoints include FVIII activity level after the onset of steady state and through 12 months following infusion of giroctocogene fitelparvovec.

About giroctocogene fitelparvovec

The U.S. Food and Drug Administration has granted Orphan Drug, Fast Track, and regenerative medicine advanced therapy (RMAT) designations to giroctocogene fitelparvovec, which also received Orphan Medicinal Product designation from the European Medicines Agency. Giroctocogene fitelparvovec is being developed as part of a collaboration agreement for the global development and commercialization of gene therapies for hemophilia A between Sangamo and Pfizer. In late 2019, Sangamo transferred the manufacturing technology and the Investigational New Drug (IND) application to Pfizer. Giroctocogene fitelparvovec is currently being studied in the Phase 3 AFFINE study.

About Hemophilia A

Hemophilia is a genetic hematological rare disease that results in a deficiency of a protein that is required for normal blood clotting — clotting factor

VIII in hemophilia A. The severity of hemophilia that a person has is determined by the amount of 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 one in every 5,000-10,000 male births worldwide. 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 Pfizer Rare Disease

Rare diseases include some of the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including rare hematologic, neurologic, cardiac and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

About Pfizer: Breakthroughs That Change Patients' Lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 170 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.Pfizer.com. In addition, to learn more, please visit us on www.Pfizer.com and follow us on Twitter at @Pfizer and @Pfizer News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

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. For more information about Sangamo, visit www.sangamo.com.

PFIZER DISCLOSURE NOTICE:

The information contained in this release is as of December 12, 2021. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about an investigational hemophilia A therapy, giroctocogene fitelparvovec (SB-525 or PF-07055480), including its potential benefits and the phase 1/2 and phase 3 clinical trials, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; whether and when the clinical hold of the Phase 3 AFFINE clinical trial will be lifted; risks associated with interim data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when drug applications for any potential indications for giroctocogene fitelparvovec may be filed in any jurisdictions; whether and when regulatory authorities in any jurisdictions may approve any such applications, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether giroctocogene fitelparvovec will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of giroctocogene fitelparvovec; uncertainties regarding the impact of COVID-19 on Pfizer's business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2020 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com.

SANGAMO DISCLOSURE NOTICE:

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements regarding the therapeutic potential of giroctocogene fitelparvovec (SB-525), including its potential clinical benefit to patients with hemophilia A and its potential as an alternative to the standard of care for patients with hemophilia A, the anticipated implementation of a protocol amendment for, and the response to the clinical hold of, the Phase 3 AFFINE study of giroctocogene fitelparvovec and the expected 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 in these forward looking statements. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the evolving COVID-19 pandemic and its impact on the global business environment, healthcare systems and the business and operations of Sangamo and Pfizer, including the initiation and operation of clinical trials; the research and development process; the uncertain timing and unpredictable nature of clinical trial results, including the risk that any protocol amendment for the Phase 3 AFFINE trial of giroctocogene fitelparvovec may not be accepted by the relevant review bodies in

a timely manner, or at all, each of which could further delay or preclude further patient dosing in the trial, as well as the risk that therapeutic effects observed in the preliminary results of the Phase 1/2 Alta study will not be durable in patients and that final clinical trial data will not validate the safety and efficacy of giroctocogene fitelparvovec; reliance on results of early clinical trials, such as the Phase 1/2 Alta study, which results are not necessarily predictive of future clinical trial results, including the results in the Phase 3 AFFINE study; 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 and Pfizer in giroctocogene fitelparvovec; the potential for Pfizer to terminate the giroctocogene fitelparvovec program or to breach or terminate its collaboration agreement with Sangamo; and the potential for Sangamo to fail to realize its expected benefits of its collaboration with Pfizer, including the risk that Sangamo may not earn any additional milestone or royalty payments under its collaboration with Pfizer. These risks and uncertainties are described more fully in Sangamo's filings with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K for the year ended December 31, 2020 and the most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2021. The information contained in this release is as of December 12, 2021, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.

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