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# Pfizer and Sangamo Announce Updated Phase 1/2 Results Showing Sustained Factor VIII Activity Levels and No Bleeding Events or Factor Usage in 3e13 vg/kg Cohort Following giroctocogene fitelparvovec (SB-525) Gene Therapy

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*— Dosing of the first patient in the pivotal Phase 3 study anticipated in second half of 2020 —*

NEW YORK & BRISBANE, Calif.--(BUSINESS WIRE)-- Pfizer Inc. (NYSE:PFE)andSangamo Therapeutics, Inc. (Nasdaq:SGMO), a genomic medicines company, today announced updated follow-up data from the Phase 1/2 Alta study of giroctocogene fitelparvovec (SB-525, or PF-07055480), an investigational gene therapy for patients with severe hemophilia A. All five patients with severe hemophilia A who received the 3e13 vg/kg dose showed sustained factor VIII (FVIII) activity levels, with a median of 64.2% via chromogenic assay (patient-level geometric means after week 9 post-infusion). No patients experienced bleeding events or required FVIII infusions. The factor VIII activity levels reflect measurements up to 61 weeks, the extent of follow-up for the longest-treated patient in the cohort. These data are being presented today as a late-breaking oral abstract at the World Federation of Hemophilia 2020 World Congress, which is being held virtually from June 14 to June 19, 2020.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20200618005091/en/

Giroctocogene fitelparvovec was generally well tolerated. As previously reported, one patient in the 3e13 vg/kg 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 which fully resolved within 24 hours. No other treatment-related serious adverse events were reported. Among the five patients in the 3e13 vg/kg dose cohort, four received corticosteroids for liver enzyme (alanine aminotransferase, ALT) elevations. Three patients had subsequent ALT elevations that responded to corticosteroids. All episodes of ALT elevations fully resolved with oral corticosteroids.

“We are excited that these data affirm previous findings from this Phase 1/2 study, and that all five patients have sustained levels of factor VIII activity with no bleeding events or use of factor replacement therapy. We are encouraged by the potential of giroctocogene fitelparvovec to demonstrate longer-term durability, an important element for patients living with severe hemophilia A,” said Seng Cheng, Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Disease Research Unit. “The Phase 3 lead in study is ongoing, and we look

forward to dosing patients with this investigational gene therapy in the pivotal Phase 3 trial later this year."

"The current standard of care for severe hemophilia A requires regular infusions to replace missing Factor VIII. Gene therapy, on the other hand, offers a new approach with the potential to provide a one-time treatment that would enable patients to produce the missing factor on their own," said Bettina M. Cockroft, M.D., M.B.A., Chief Medical Officer of Sangamo. "These follow-up data indicate that treatment with giroctocogene fitelparvovec resulted in sustained factor levels up to 14 months following treatment and suggests the potential of this investigational gene therapy to alleviate the treatment burden of current hemophilia disease management."

The additional follow-up builds on data presented at the 61st Annual Meeting of the American Society of Hematology (ASH) in December 2019, which demonstrated that giroctocogene fitelparvovec was generally well tolerated and resulted in sustained FVIII levels up to 44 weeks, the extent of follow-up for the longest-treated patient in the 3e13 vg/kg dose cohort at that time. The previously presented data included 11 patients treated across four ascending dose cohorts: 9e11 vg/kg (2 patients), 2e12 vg/kg (2 patients), 1e13 vg/kg (2 patients) and 3e13 vg/kg (5 patients). Pfizer and Sangamo plan to present further follow-up data from the Alta study when all five patients in the 3e13 vg/kg dose cohort have been followed for at least one year.

**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 patients assessed across four dose cohorts is 30 years (range 18-47 years). All 11 patients are male. 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.

**About giroctocogene fitelparvovec**

Giroctocogene fitelparvovec (SB-525 or PF-07055480), comprises a recombinant adeno-associated virus serotype 6 vector (AAV6) encoding the complementary deoxyribonucleic acid for B domain deleted human FVIII. The giroctocogene fitelparvovec expression cassette was designed for optimal liver-specific expression of FVIII protein and supports production of high yields of the vector. The giroctocogene fitelparvovec transcriptional cassette incorporates multi-factorial modifications to the liver-specific promoter module, FVIII transgene, synthetic polyadenylation signal and vector backbone sequence.

In late 2019, Sangamo transferred the manufacturing technology and the Investigational New Drug (IND) application to Pfizer. Pfizer is enrolling patients in the Phase 3 lead-in study (ClinicalTrials.gov Identifier: NCT03587116), the data from which is expected to provide a baseline for patients who are subsequently enrolled into the pivotal Phase 3 study (ClinicalTrials.gov Identifier: NCT04370054). The primary endpoint of the Phase 3 study is annualized bleeding rate (ABR) over 12 months, and secondary endpoints include steady state FVIII activity levels, annualized infusion rate of exogenous FVIII activity, annualized FVIII consumption, ABR and total ABR of specific type by cause and by location, and change in joint health using Hemophilia Joint Health Score, over 12 months.

**About Sangamo Therapeutics**

Sangamo Therapeutics is committed to translating ground-breaking science into genomic medicines with the potential to transform patients' lives using gene therapy, *ex vivo* gene-edited cell therapy, and *in vivo* genome editing and gene regulation. For more information about Sangamo, visit www.sangamo.com.

**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.

**Pfizer Inc.: 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 150 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.

***SANGAMO DISCLOSURE NOTICE:***

*This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to the potential to develop, obtain regulatory approvals for and commercialize SB-525 as a safe and effective therapy to treat hemophilia A, the potential long-term durability of SB-525 therapy, anticipated plans and timelines for conducting phase 3 clinical trials and sharing additional clinical data 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. There can be no assurance that Sangamo will earn any additional milestone or royalty payments under the Pfizer collaboration. 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; the research and development process; the uncertain timing and unpredictable results of clinical trials, including whether final clinical trial data will validate the safety and efficacy of SB-525; 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 SB-525; the potential for Pfizer to terminate the SB-525 program or to breach or terminate its collaboration agreement with Sangamo; and the potential for Sangamo for fail to realize its expected benefits of 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 most recent Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 and Annual Report on Form 10-K for the year ended December 31, 2019. The information contained in this release is as of June 18, 2020, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.*

***PFIZER DISCLOSURE NOTICE:***

*The information contained in this release is as of June 18, 2020. 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 anticipated timing of a phase 3 clinical trial, 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; 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, 2019 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.*

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