Pfizer and Sangamo Dose First Participant in Phase 3 Study Evaluating Hemophilia A Gene Therapy Treatment

October 7, 2020

NEW YORK & BRISBANE, Calif.--(BUSINESS WIRE)--Oct. 7, 2020-- Pfizer Inc. (NYSE: PFE) and Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicines company, today announced that the first participant has been dosed in the Phase 3 AFFINE study of giroctocogene fitelparvovec (SB-525), an investigational gene therapy for hemophilia A patients.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20201007005049/en/

AFFINE is a global Phase 3, open-label, multicenter, single arm study that will evaluate the efficacy and safety of giroctocogene fitelparvovec in patients with moderately severe to severe hemophilia A. The primary endpoint is impact on annual bleed rate (ABR) through 12 months following treatment with giroctocogene fitelparvovec, compared to ABR on Factor VIII (FVIII) replacement therapy collected in the Phase 3 lead-in study period. Participants will be analyzed throughout the 5-year study period following the single infusion to further assess the durability and efficacy.

“The initiation of the pivotal Phase 3 dosing study of giroctocogene fitelparvovec is a significant achievement for Pfizer as we continue our longstanding commitment to improving care for the hemophilia community,” said Brenda Cooperstone, Chief Development Officer, Rare Disease, Pfizer Global Product Development. “Enrollment in the lead-in study is progressing well and recruitment is on track for Phase 3. Given the Phase 1/2 study findings to date, we believe that giroctocogene fitelparvovec has the potential to sustain factor levels and reduce annual bleed rates, suggesting this one-time gene therapy could potentially transform the standard of care for eligible patients worldwide.”

Data from the Phase 3 lead-in study will provide a baseline for patients evaluated in the Phase 3 study. Updated Phase 1/2 data announced at a Pfizer investor event on September 15, 2020 demonstrated that giroctocogene fitelparvovec was generally well tolerated. Each of the five patients in the high dose cohort sustained FVIII activity levels without bleeds or the need for prophylactic factor through up to 85 weeks. Factor VIII activity levels were sustained at a clinically meaningful level, with a geometric mean of ~71% when measured between the weeks of 9 and 52.

“We are encouraged that findings from the Phase 1/2 Alta study met two critically important measures for the hemophilia A patient community, showing clinically meaningful factor levels and reduced bleeds,” said Bettina M. Cockroft, M.D., M.B.A, Chief Medical Officer of Sangamo. “The progress of this program, the most advanced of our gene therapy product candidates, into Phase 3 is an important milestone for Sangamo, as it represents our first asset in a registrational trial.”

Per the terms of the collaboration agreement, Sangamo has now earned a $30 million milestone payment. The giroctocogene fitelparvovec collaboration was established in May 2017. Under the terms, Pfizer is now operationally and financially responsible for research, development, manufacturing and commercialization activities for giroctocogene fitelparvovec following the transfer of the Investigational New Drug (IND) from Sangamo to Pfizer in December 2019. Sangamo is eligible to receive total potential milestone payments of up to $300 million for the development and commercialization of giroctocogene fitelparvovec, and up to $175 million for additional hemophilia A gene therapy product candidates that may be developed under the collaboration. Sangamo will, additionally, receive tiered royalties starting in the low teens and up to 20% of annual net sales of giroctocogene fitelparvovec.

About the AFFINE study

The Phase 3 AFFINE (effic**A**cy and sa**F**ety **F**actor v**I**i ge**N**e th**E**rapy in hemophilia A patients; 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 ABR through 12 months following treatment with giroctocogene fitelparvovec, as compared to ABR on prior FVIII prophylaxis replacement therapy. The secondary endpoint is FVIII activity level after the onset of steady state and through 12 months following infusion of giroctocogene fitelparvovec.

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.

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 global collaboration between Sangamo and Pfizer.

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 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 disease includes some of the most serious of all illnesses and impacts 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, the potential for Sangamo to earn milestone payments and royalties under its collaboration with Pfizer and the timing of such payments and royalties 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 June 30, 2020 and Annual Report on Form 10-K for the year ended December 31, 2019. The information contained in this release is as of October 7, 2020, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.

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The information contained in this release is as of October 7, 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, 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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