



# Pfizer and Sangamo Announce Updated Phase 1/2 Results Showing Sustained Factor VIII Activity Levels in $3 \times 10^{13}$ VG/KG Cohort Through One Year Following Hemophilia A Gene Therapy

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- First patient was dosed in pivotal Phase 3 AFFINE study in October 2020

NEW YORK & BRISBANE, Calif.--(BUSINESS WIRE)-- 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 (SB-525 or PF-07055480), an investigational gene therapy for patients with severe hemophilia A. These data are being presented today at the 62nd American Society for Hematology Annual meeting taking place virtually from December 5th - 8th. The oral presentation slides, which include follow-up data up to 85 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/20201207005251/en/>

All five patients in the high dose  $3 \times 10^{13}$  vg/kg cohort have had at least one year of follow-up and showed sustained factor VIII (FVIII) activity levels, with a group median FVIII activity of 56.9% and a group geometric mean FVIII activity of 70.4% via chromogenic

assay from week 9 to 52. Steady-state FVIII activity was achieved for all patients in the 3 x 10<sup>13</sup> vg/kg cohort within 9 weeks of treatment with giroctocogene fitelparvovec, with no bleeding events and no FVIII infusions (beyond 3 weeks post-infusion) within the first year. As of the cutoff date of August 31, 2020, one patient had one target joint bleed requiring FVIII therapy, occurring after week 52.

“It is promising to see how quickly all five patients in the 3 x 10<sup>13</sup> vg/kg cohort achieved steady-state FVIII activity levels, with no bleeding events and no factor usage within the first year and only one target joint bleed after 52 weeks,” said Andrew D. Leavitt, MD, Professor of Medicine, University of California, San Francisco, CA, and investigator of the Alta and AFFINE studies. “Our focus now is to confirm these exciting findings in the Phase 3 study, and to gather long-term data by following these patients and others in the Phase 3 study over a longer period of time.”

Giroctocogene fitelparvovec was generally well tolerated. As previously reported, one patient in the 3 x 10<sup>13</sup> 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 as of the cutoff date. Among the five patients in the 3 x 10<sup>13</sup> 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, and as of the cutoff date no participants were on corticosteroids and no corticosteroid use has been initiated after week 52.

“We continue to be encouraged by the findings from this Phase 1/2 study, which now include durable factor VIII expression through one year of follow-up, and we look forward to continuing to follow these patients,” said Seng Cheng, Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Disease Research Unit. “With the first patient dosed in the Phase 3 AFFINE study in October 2020, we are on track for a readout from this pivotal Phase 3 trial in 2022, which will allow us to better assess the potential of our gene therapy across a larger sample size.”

“These latest results demonstrate that this gene therapy may bring clinical benefit to patients and has the potential to serve as an alternative to the burdensome standard of care for patients with hemophilia A,” said Bettina Cockroft, M.D., M.B.A, Chief Medical Officer of Sangamo. “We look forward to continuing to support our collaboration partners at Pfizer as they conduct the Phase 3 AFFINE study and assess the full potential of this

promising therapy.”

Pfizer and Sangamo plan to present further follow-up data from the Alta study when all five patients in the 3 x 10<sup>13</sup> vg/kg dose cohort have been followed for at least two years.

#### 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 (9x10<sup>11</sup> vg/kg - 2 patients, 2 x 10<sup>12</sup> vg/kg - 2 patients, 1x10<sup>13</sup> vg/kg - 2 patients and 3 x 10<sup>13</sup> vg/kg - 5 patients) is 30 years (range 18-47 years). After one year of follow-up for all patients in the study, participants will be assessed every 6 months until they enroll into 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.

## 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.

## 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](http://www.pfizer.com). In addition, to learn more, please visit us on [www.pfizer.com](http://www.pfizer.com) and follow us on Twitter at @Pfizer and @Pfizer\_News, LinkedIn, YouTube and like us on Facebook at [www.facebook.com/Pfizer](http://www.facebook.com/Pfizer).

### 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 genome regulation. For more information about Sangamo, visit [www.sangamo.com](http://www.sangamo.com).

### PFIZER DISCLOSURE NOTICE:

The information contained in this release is as of December 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 and the anticipated timing and readout of the 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](http://www.sec.gov) and [www.pfizer.com](http://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 relating to 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 readout from the Phase 3 AFFINE study and the expected timing thereof, the plan and related timelines for 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 nature of clinical trial results, including the risks that therapeutic effects observed in clinical trial results 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 most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2020. The information contained in this release is as of December 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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Source: Pfizer Inc.