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Sangamo and Pfizer Announce Updated Phase 1/2 Results Showing Sustained Increased Factor VIII Activity Through 44 Weeks Following SB-525 Gene Therapy Treatment

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- The first two patients treated with the 3e13 vg/kg dose achieved stable Factor VIII (FVIII) levels demonstrating durability in the normal range through 44 and 37 weeks, respectively - All five patients in the 3e13 vg/kg dose cohort achieved normal range FVIII levels within 5-7 weeks following treatment, with no bleeding events with up to 44 weeks of follow-up - Lower-dose cohorts indicated durable FVIII activity with up to 52 weeks of follow-up - The Companies have progressed SB-525 into a Phase III registrational program led by Pfizer

BRISBANE, Calif. & NEW YORK--(BUSINESS WIRE)--Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, and Pfizer, Inc. (NYSE: PFE), today announced updated follow-up results from the Phase 1/2 Alta study evaluating investigational SB-525 gene therapy in patients with severe hemophilia A. The data showed that SB-525 was generally well tolerated and demonstrated sustained increased Factor VIII (FVIII) levels following treatment with SB-525 through to 44 weeks, the extent of follow-up for the longest treated patient in the 3e13 vg/kg dose cohort. Data from 11 patients treated with SB-525 will be featured in a poster presentation today, December 7, 2019, at the 61st Annual Meeting of the American Society of Hematology (ASH) in Orlando, FL. The SB-525 ASH poster, which includes the full set of data, is available on Sangamo's website in the Investors and Media section under Events and Presentations.

"I am pleased that all five patients in the high dose (3e13 vg/kg) cohort rapidly achieved normal levels of Factor VIII, and that Factor VIII levels have been stable and durable in the normal range for the first two patients up to 44 and 37 weeks following treatment respectively, with no bleeding events or factor usage up to a follow up of 44 weeks in the longest treated patient," said Barbara Konkle, M.D., Bloodworks Northwest, Professor of Medicine at University of Washington and a Principal Investigator of the Alta study. "It is important to continue to follow these patients to determine whether these results are sustained in the longer term as the combination of a favorable safety profile coupled with sustained expression at a level that prevents bleeding and allows normal activity will be the hallmark of a successful gene therapy for hemophilia A."

Alta study data presented at ASH 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). The data cutoff date was October 17, 2019.

An analysis of plasma FVIII antigen was assessed by ELISA and demonstrated antigen concentrations consistent with the FVIII activity measured by the chromogenic assay. Dose dependent increases in FVIII activity over baseline were observed across the dose cohorts. The lower-dose cohorts indicate durable FVIII activity with up to 52 weeks of follow-up.

In the 3e13 vg/kg dose cohort, patients achieved normal range FVIII activity within 5-7 weeks of treatment with SB-525. The first two patients treated in this cohort (Patients 7 and 8) have achieved stable FVIII levels, demonstrating durability in the normal range through 44 and 37 weeks, respectively, as measured by the chromogenic assay. The two patients most recently treated in this cohort (Patients 10 and 11), with 22 and 12 weeks of follow-up, respectively, demonstrated a similar pattern of FVIII expression. The FVIII expression pattern observed in Patient 9 differed from that of other patients in the cohort. Seven weeks following treatment, Patient 9 achieved normal range FVIII levels. Beginning at week 13, FVIII levels in that patient fluctuated in a range below normal, but still well above the level needed to prevent spontaneous bleeding. At week 18, FVIII levels in Patient 9 began to increase, and as of the latest measurement at week 24, continued to rise. No patient in the 3e13 vg/kg dose cohort has experienced bleeding events up to 44 weeks of follow-up, and no patient in this dose cohort required factor replacement following initial use of prophylactic factor.

SB-525 was generally well tolerated across all dose cohorts. The treatment-related adverse events include: alanine aminotransferase (ALT) elevation (36.4%, n=4), pyrexia (27.3%, n=3), increased aspartate aminotransferase (18.2%, n=2), tachycardia (18.2% n=2), fatigue (9.1%, n=1), hypotension (9.1%, n=1) and myalgia (9.1%, n=1). Treatment-related serious adverse events (SAEs) of hypotension (grade 3) and fever (grade two) occurred in one patient in the 3e13 vg/kg cohort six hours following dosing with SB-525 that fully resolved within 24 hours. No similar events were reported in the other patients dosed in that cohort. No patients treated with SB-525 experienced an ALT elevation associated with loss of Factor VIII expression. In the 3e13 vg/kg dose cohort, four patients experienced transient low grade ALT elevations (>1.5 x baseline) that were managed with a tapering course of oral steroids. The study does not use corticosteroids prophylactically, initiating them only in the event of an ALT elevation that is greater than 1.5x baseline.

“The updated results from the Alta study suggest that SB-525 may represent a differentiated gene therapy for patients with severe hemophilia A,” said Bettina Cockroft, M.D., Chief Medical Officer of Sangamo. “The results continue to suggest that if sustained over a longer duration, SB-525 has the potential to be a predictable, reliable, and safe treatment that may bring clinical benefits to patients with severe hemophilia A.”

Sangamo has completed the manufacturing technology transfer and initiated the transfer of the Investigational New Drug (IND) Application to Pfizer, which is expected to be completed in the first quarter 2020. Pfizer is enrolling patients in the Phase 3 lead-in study, the data from which is expected to provide a baseline for patients who are subsequently enrolled into the Phase 3 study (ClinicalTrials.gov Identifier: NCT03587116).

“We are pleased with the progress that we have made in progressing SB-525 gene therapy toward a Phase 3 registrational study, including enrolling the first patient in the 6-month lead-in study. We expect to dose the first patient in the Phase 3 registrational study in 2020,” said Seng Cheng, Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Diseases Research Unit. “We continue to believe that if the observed safety and efficacy results are sustained, this gene therapy has the potential to transform the treatment paradigm of severe hemophilia A.”

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 SB-525 in patients with severe hemophilia A. The mean age of the 11 patients assessed 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 SB-525, which also received Orphan Medicinal Product designation from the European Medicines Agency. SB-525 is being developed as part of a global collaboration between Sangamo and Pfizer.

About SB-525 Gene Therapy

SB-525 comprises a recombinant adeno-associated virus serotype 6 vector (AAV6) encoding the complementary deoxyribonucleic acid for B domain deleted human FVIII. The SB-525 vector cassette was designed to optimize both the vector manufacturing yield and liver-specific FVIII protein expression. The SB-525 transcriptional cassette incorporates multi-factorial modifications to the liver-specific promoter module, FVIII transgene, synthetic polyadenylation signal and vector backbone sequence.

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.

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Sangamo 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 the investigational hemophilia A gene therapy, SB-525, including its potential therapeutic benefits; the potential long-term durability of SB-525 gene therapy; SB-525 having the potential to be a predictable and reliable treatment that may bring clinical benefit to patients with hemophilia A and to potentially represent a transformative treatment paradigm; plans to advance SB-525 into a potential registrational study; the potential benefits of the RMAT and Orphan medicine designation for SB-525; and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to risks and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the research and development process; additional data, including the risk that the data reported from the Alta to date may not be indicative of the final results from the Alta study or that such final results may not validate and support the safety and efficacy of SB-525; the completion of the Alta study; the possibility of unfavorable new clinical data from the Alta study and further analyses of existing clinical data from the study that may material change clinical outcomes; 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 the clincal studies relating to SB-525, any potential registrational studies or any other clinical studies of SB-525; whether Sangamo will be able to maintain or receive the benefits associated with RMAT, Orphan Drug, Fast Track and Orphan Medicinal Product designations for SB-525; Sangamo's reliance on Pfizer and other third-parties to meet their clinical and manufacturing obligations; Sangamo's ability to maintain strategic partnerships; and the potential for technological developments by Sangamo's competitors that will obviate Sangamo's gene therapy technology. Further, there can be no assurance that the necessary regulatory approvals will be obtained for SB-525 or that Sangamo and its partners will be able to develop commercially viable

product candidates. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations and business environments. These risks and uncertainties are described more fully in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the Securities and Exchange Commission and Sangamo's most recent Quarterly Report on Form 10-Q. Forward-looking statements contained in this announcement are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

Pfizer Disclosure Notice: The information contained in this release is as of December 7, 2019. 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 agent, SB-525, 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 SB-525 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 SB-525 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 SB-525; 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, 2018 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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