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SANGAMO ANNOUNCES POSITIVE PRELIMINARY DATA FROM THE PHASE 1/2 ALTA STUDY EVALUATING SB-525 GENE THERAPY FOR HEMOPHILIA A

Wednesday, August 08, 2018 - 08:00am

Richmond, California, August 8, 2018 – Sangamo Therapeutics, Inc. (Nasdaq: SGMO) today announced positive preliminary data from the Phase 1/2 clinical trial evaluating SB-525, a cDNA gene therapy candidate for Hemophilia A (the "Alta study"). SB-525 is being developed as part of a global collaboration between Sangamo and Pfizer Inc. for the development and commercialization of potential gene therapy programs for Hemophilia A.

The Alta study is an open-label, dose-ranging clinical trial designed to assess the safety and tolerability of SB-525 in up to 20 adult subjects with severe Hemophilia A. To date, five patients have been treated at three dose levels. A sixth patient is scheduled for treatment later this month. During the initial dose escalation phase, this study enrolls two patients per dose cohort.

Preliminary Observations

• In the Alta study, SB-525 has been generally well tolerated to date with no treatmentrelated serious adverse events and no use of tapering courses of oral steroids.
• The fifth patient in the study, the first at the third dose level, was treated in June and has achieved therapeutic Factor VIII activity levels.*
• A dose dependent effect has been observed in the study, with patients in the second dose cohort reporting reduced use of factor replacement.

Sangamo and Pfizer expect to present detailed data from the Alta study at a hematology conference in the fourth quarter.

"We have made good progress with dose escalation in this study and are encouraged by the safety and tolerability profile to date and by the attainment of therapeutic Factor VIII activity levels in the first patient in the third dose cohort," said Edward Conner, MD, Chief Medical Officer of Sangamo. "We look forward to generating additional data to assess the consistency and sustainability of the Factor VIII expression observed."

*Epidemiological data indicate that Factor VIII activity above 12% of normal is associated with substantial reduction or elimination of spontaneous bleeds and factor usage. Den Uijl IE et al Haemophilia 2011; 17(6):849-53

About SB-525
SB-525 comprises a recombinant adeno-associated virus (rAAV) vector carrying a Factor VIII gene construct driven by a proprietary, synthetic, liver-specific promoter. The U.S. Food and Drug Administration has granted Orphan Drug and Fast Track designations to SB-525, which also received Orphan Medicinal Product designation from the European Medicines Agency. Hemophilia A is a rare blood disorder caused by a genetic mutation resulting in insufficient activity of Factor VIII, a blood clotting protein the body uses to stop bleeding. There are approximately 16,000 patients in the U.S. and more than 150,000 worldwide with Hemophilia A.

About Sangamo
Sangamo Therapeutics is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the Company's platform technologies in genome editing, gene therapy, gene regulation and cell therapy. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements
This press release contains forward-looking statements regarding Sangamo's current expectations. These forward looking statements include, without limitation, statements related to the potential for SB-525 to treat Hemophilia A, the expectation of generating additional data to assess the consistency and sustainability of the FVIII expression observed, the importance of consistency and sustainability of Factor VIII expression, and Sangamo's expectation that it will present detailed data from the Alta study at a hematology conference in the fourth quarter of 2018, and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks, uncertainties 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 completion of the Alta study, the fact that the preliminary observations from the Alta study are based on preliminary data from only the first five patients in the study and that these preliminary data may not be representative of final results after all patients are treated in the study and all data are collected and analyzed; whether the final results from the Alta study will validate and support the safety and efficacy of SB-525, including the risk that the observed therapeutic Factor VIII activity levels and reduced use of factor replacement in the Alta study to date may not be maintained or replicated, Sangamo's reliance on Pfizer and other third-parties to meet their clinical and manufacturing obligations, and the ability to maintain strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals will be obtained 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 these and other risks and uncertainties that exist in Sangamo's operations and business environments. These risks and uncertainties are described more fully in Sangamo's Quarterly Report on Form 10-Q for the quarter ended March 31, 2018, as filed with the Securities and Exchange Commission. Forwardlooking 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.

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