Pfizer Logo

Sangamo Receives Fast Track Designation From The Fda For Sb-525 Investigational Hemophilia A Gene Therapy

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Sangamo Therapeutics, Inc. (NASDAQ: SGMO) announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to SB-525, the Company's clinical stage cDNA gene therapy candidate for hemophilia A, which is being developed as part of an exclusive, global collaboration and license agreement with Pfizer Inc. (NYSE: PFE). The FDA's Fast Track designation is designed to facilitate the development and expedite the review of drugs and biologics to treat serious conditions and fill an unmet medical need. Once a drug receives Fast Track designation, early and frequent communication with the FDA is encouraged throughout the development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

SB-525 uses a recombinant adeno-associated virus (rAAV) to deliver a human Factor VIII cDNA construct and proprietary, synthetic liver-specific promoter to the nucleus of liver cells with a single infusion. The therapy is designed as a single treatment strategy intended to provide continuous, therapeutic expression of Factor VIII protein.

SB-525 has already received Orphan Drug designation from the FDA. The FDA has cleared an Investigational New Drug application for this program, and a Phase 1/2 clinical trial evaluating SB525 in adults with hemophilia A is expected to open and begin screening subjects for enrollment by the end of the second quarter 2017. Data from this study are expected in late 2017 or early 2018.

About Hemophilia A

Hemophilia A is a monogenic, rare bleeding disorder in which the blood does not clot normally. It is caused by mutations in the F8 gene which encodes Factor VIII clotting protein that helps the blood clot and stop bleeding when blood vessels are injured. Individuals with this mutation experience bleeding episodes after injuries and spontaneous bleeding episodes that often lead to joint disease such as arthritis. According to the Centers for Disease Control and Prevention, hemophilia occurs in about one of every 5,000 male births, with an estimated 20,000 males in the U.S. living with the disorder.

About Sangamo Therapeutics

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company is advancing Phase 1/2 clinical programs in Hemophilia A and Hemophilia B, and lysosomal storage disorders MPS I and MPS II. Sangamo has an exclusive, global collaboration and license agreement with Pfizer Inc. for gene therapy programs for Hemophilia A, with Bioverativ Inc. for San 510-97 hemoglobinopathies, including beta thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, it has established

strategic partnerships with companies in non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at www.sangamo.com.

Forward Looking Statements

This press release may contain forward-looking statements based on Sangamo’s current expectations. These forward-looking statements include, without limitation references relating to the benefit of Fast Track designation to accelerate regulatory approval of SB-525, research and development of therapeutic applications of Sangamo's gene therapy and ZFP technology platforms, the potential of Sangamo's technology to treat hemophilia and lysosomal storage disorders, and the expected timing of initiating clinical trials of SB-525 and the release of data from these trials. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to substantial dependence on the clinical success of lead therapeutic programs, the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of ZFNs, technological challenges, Sangamo’s ability to develop commercially viable products and technological developments by our competitors. For a more detailed discussion of these and other risks, please see Sangamo’s SEC filings, including the risk factors described in its Annual Report on Form 10-K and its most recent Quarterly Report on Form 10-Q. Sangamo Therapeutics, Inc. assumes no obligation to update the forward-looking information contained in this press release.

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