Sangamo Therapeutics Logo

Sangamo BioSciences Receives Orphan Drug Designation From The FDA For SB-FIX, The First Application Of Therapeutic In Vivo Genome Editing

September 6, 2016

RICHMOND, Calif., Sept. 6, 2016 /PRNewswire/ -- Sangamo BioSciences, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to SB-FIX, the company's zinc finger nuclease (ZFN)-mediated genome editing product candidate for the treatment of hemophilia B. Sangamo expects to initiate a Phase 1/2 clinical study (SB-FIX-1501) in adult subjects with the disease in 2016.

"We are pleased with the FDA's decision to grant orphan drug designation to SB-FIX for hemophilia B," said Geoff Nichol, M.B., Ch.B., Sangamo's executive vice president of research and development. "By enabling targeted integration of a therapeutic factor 9 gene, our ZFN-mediated genome editing approach may offer hemophilia B patients a therapeutic option that has potential advantages over conventional gene therapy approaches. We will enroll adult hemophilia patients into our first clinical trial, however, our goal is to move into pediatric patients, a population we believe could particularly benefit from a treatment that has the potential to provide lifelong expression of therapeutic levels of Factor IX protein."

"Our mission is to translate our ground-breaking science into genetic therapies that transform patients' lives," said Sandy Macrae, M.B., Ch.B., Ph.D., Sangamo's president and chief executive officer. "This is another positive step forward for the first therapeutic *in vivo* genome editing application cleared for clinical evaluation in humans. I am pleased with the progress that we have made to advance this program and we look forward to initiating a Phase 1/2 clinical trial by the end of 2016."

The FDA grants orphan drug designation to investigational drugs and biologics that are intended for the treatment of rare diseases that affect fewer than 200,000 people in the U.S. Orphan drug status is intended to facilitate drug development for rare diseases and provides several benefits to drug developers, including assistance with clinical study design and drug development, tax credits for qualified clinical trials costs, exemptions from certain FDA application fees, and seven years of market exclusivity upon regulatory product approval.

Sangamo's *In Vivo* Genome Editing Approach
SB-FIX is designed as a single treatment strategy intended to provide stable, continuous production of Factor IX clotting protein (FIX) for the lifetime of the patient. Sangamo's ZFN-mediated *in vivo* genome editing approach makes use of the albumin gene locus, a highly expressing and liver-specific genomic "safe-harbor site," that can be edited with ZFNs to accept and express therapeutic genes. The approach is designed to enable the patient's liver to permanently produce circulating therapeutic levels of a corrective protein product. The ability to permanently integrate the therapeutic gene in a highly specific targeted fashion significantly differentiates Sangamo's *in vivo* genome editing approach from conventional AAV cDNA gene therapy approaches, which are non-integrating, and may "wash out" of the liver as cells divide and turn over. Ultimately, the target population for these programs will include pediatric patients and it will be important in this population to be able to produce stable levels of therapeutic protein for the lifetime of the patient. With such a large capacity for protein production (approximately 15g/day of albumin), targeting and co-opting only a very small percentage of the albumin gene's capacity is sufficient to produce the needed replacement protein at therapeutically relevant levels with no significant effect on albumin production.

About Hemophilia B
Hemophilia, a rare bleeding disorder in which the blood does not clot normally, is caused by mutations in genes that encode factors which help the blood clot and stop bleeding when blood vessels are injured. Hemophilia B is caused by a defect in the gene encoding clotting Factor IX protein and 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 National Hemophilia Foundation and the World Federation of Hemophilia, hemophilia B occurs in about one in every 50,000 male births with approximately 4,000 males currently affected in the U.S. The current standard treatment for individuals with hemophilia is protein replacement of the defective clotting factor with regular infusion of recombinant clotting factors or plasma concentrates. These therapies are expensive and sometimes stimulate the body to produce antibodies that inhibit the benefits of treatment. The most severe forms of hemophilia B require the need for ongoing, preventive infusions.

About Sangamo
Sangamo BioSciences, Inc. is focused on Engineering Genetic Cures® for monogenic and infectious diseases by deploying its novel zinc finger DNA-binding protein technology, in therapeutic genome editing and gene regulation, and AAV-based gene therapy platforms. The Company's proprietary ZFN-mediated *in vivo* genome editing approach is focused on monogenic diseases, including hemophilia and lysosomal storage disorders. Based on its *in vivo* genome editing approach, Sangamo is initiating a Phase 1/2 clinical trial for hemophilia B, the first *in vivo* genome editing application cleared by the FDA. In addition, Sangamo has a Phase 2 clinical program to evaluate the safety and efficacy of novel ZFP Therapeutics® for the treatment of HIV/AIDS (SB-728). The Company has also formed a strategic collaboration with Biogen Inc. for hemoglobinopathies, such as sickle cell disease and beta-thalassemia, and with Shire International GmbH to develop therapeutics for Huntington's disease. It has established strategic partnerships with companies in non-therapeutic applications of its technology, including Dow AgroSciences and Sigma-Aldrich Corporation. For more information about Sangamo, visit the Company's website at www.sangamo.com.

ZFP Therapeutic® is a registered trademark of Sangamo BioSciences, Inc.

This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include,

without limitation, references relating to research and development of novel ZFP TFs and ZFNs and therapeutic applications of Sangamo's ZFP technology platform, the potential of Sangamo's ZFP technology to treat hemophilia B, and lysosomal storage disorders, the expected timing of trial enrollment for SB-FIX-1501, the impact of the SB-FIX clinical trial on the field of genetic medicine, the benefit of orphan drug status, and the safety and efficacy of the approach of using ZFN-mediated genome editing. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the safety, tolerability and efficacy of ZFNs and ZFP TFs, 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 public filings with the Securities and Exchange Commission, including the risk factors described in its Annual Report on Form 10-K and its most recent Quarterly Report on Form 10-Q. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

Logo - https://photos.prnewswire.com/prnh/20130102/SF35903LOGO

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/sangamo-biosciences-receives-orphan-drug-designation-from-the-fda-for-sb-fix-the-first-application-of-therapeutic-in-vivo-genome-editing-300322600.html

SOURCE Sangamo BioSciences, Inc.

Released September 6, 2016