Sangamo Therapeutics Logo

Sangamo Therapeutics Receives Orphan Drug Designation from the FDA for SB-913 Genome Editing Treatment for MPS II

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RICHMOND, Calif., March 1, 2017 /PRNewswire/ -- Sangamo Therapeutics, 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-913, a genome editing product candidate for the treatment of Mucopolysaccharidosis Type II (MPS II), a rare lysosomal storage disorder. Orphan drug designations are granted to drugs and biologics intended to treat rare diseases with a patient population less than 200,000 in the U.S. The designation provides incentives to advance development and commercialization of rare disease drugs. Sangamo has also submitted an application to the FDA for rare pediatric disease designation for SB-913.

MPS II is caused by mutations in the gene encoding iduronate 2-sulfatase (IDS) enzyme. Using Sangamo's zinc finger nuclease (ZFN) genome editing technology, SB-913 is designed as a single treatment strategy intended to provide stable, continuous production of the IDS enzyme for the lifetime of the patient.

In 2017, Sangamo is conducting a Phase 1/2 clinical trial evaluating SB-913 as an *in vivo* genome editing treatment for MPS II. Sangamo is also conducting Phase 1/2 studies this year evaluating *in vivo* genome editing treatments SB-318 for MPS I, another rare lysosomal storage disorder, and SB-FIX for hemophilia B, a rare blood disease. Data from these studies and from a clinical trial for a fourth lead program, SB-525, a gene therapy approach for hemophilia A, are expected in late 2017 or early 2018.

Sangamo's *In Vivo* Genome Editing Approach
Sangamo's ZFN-mediated *in vivo* genome editing approach makes use of the endogenous albumin gene locus, a highly expressing and liver-specific 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. 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. 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.

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 a strategic collaboration with Bioverativ Inc. for 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 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, the expected timing of these clinical trials and the release of data from these trials, the impact of Sangamo's clinical trials on the field of genetic medicine and the benefit of orphan drug status. 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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/sangamo-therapeutics-receives-orphan-drug-designation-from-the-fda-for-sb-913-genome-editing-treatment-for-mps-ii-300415719.html

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