Sangamo Therapeutics Logo

Sangamo Receives Fast Track Designation From The FDA For SB-318 And SB-913 In Vivo Genome Editing Product Candidates For The Treatment Of MPS I And MPS II

July 13, 2017

RICHMOND, Calif., July 13, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO) announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to SB-318 and SB-913, the Company's clinical stage *in vivo* genome editing product candidates for the treatment of Mucopolysaccharidosis Type I (MPS I) and MPS II, respectively. 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 is designed to ensure that questions and issues are resolved quickly, potentially leading to earlier drug approval and access by patients.

MPS I and MPS II are caused by mutations in the genes encoding alpha-L-iduronidase (IDUA) and iduronate 2-sulfatase (IDS) enzymes, respectively. Using Sangamo's zinc finger nuclease (ZFN) genome editing technology, SB-318 (for MPS I) and SB-913 (for MPS II) are designed as a single treatment strategy intended to provide stable, continuous production of the IDUA or IDS enzyme for the lifetime of the patient.

SB-318 and SB-913 have already received Orphan Drug and Rare Pediatric Disease designations from the FDA. The FDA has cleared an Investigational New Drug application for these programs, and Phase 1/2 clinical trials evaluating SB-318 and SB-913 in adults with MPS I and MPS II, respectively, are open and screening subjects for enrollment.

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 has open Phase 1/2 clinical trials 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 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 contains forward-looking statements, including, but not limited to, statements related to the potential therapeutic applications of, and target populations for, Sangamo's gene therapy and ZFP technology platforms, including the potential of Sangamo's technology to treat hemophilia and lysosomal storage disorders, the potential benefits of Fast Track designation, and other statements that are not historical facts. These forward-looking statements are based on Sangamo's current plans, objectives, estimates, expectations and intentions and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation, risks and uncertainties associated with: the lengthy and uncertain regulatory approval process, including the risks that Fast Track designation may not lead to an expedited development, review or approval process, and that such designation does not increase the likelihood that Sangamo's product candidates will receive regulatory approval; Sangamo's substantial dependence on the clinical success of its lead therapeutic programs; the initiation, enrollment and completion of stages of its clinical trials; whether Sangamo's 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 its competitors. A more detailed discussion of these and other risks and uncertainties may be found under the caption "Risk Factors" and elsewhere in Sangamo's SEC filings and reports, including Sangamo's Quarterly Report on Form 10-Q for the quarter ended March 31, 2017 and future filings and reports by Sangamo. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

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Released July 13, 2017