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FDA clears Sangamo BioSciences' SB-318 IND application for treatment of MPS I

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Sangamo BioSciences, Inc. (NASDAQ: SGMO), the leader in therapeutic genome editing, announced that the U.S. Food and Drug Administration (FDA) has cleared the Company's Investigational New Drug (IND) application for SB-318, a single treatment strategy intended to provide a life-long therapy for Mucopolysaccharidosis Type I (MPS I). The SB-318 IND application is now active and enables Sangamo to initiate a Phase 1/2 clinical study (SB-318-1502) designed to assess the safety, tolerability and potential efficacy of SB-318 in adults with varying severities of MPS I.

SB-318 is Sangamo's second *in vivo* genome editing application cleared by the FDA; the first being for hemophilia B (SB-FIX). Both programs are based on Sangamo's proprietary In Vivo Protein Replacement Platform (IVPRP™), a single treatment strategy designed to produce stable circulating levels of a therapeutic protein from a patient's liver for the lifetime of the individual. SB-318 treatment is intended to eliminate the need for enzyme replacement therapy (ERT) which is the current standard of care for the majority of patients with MPS I. ERT for MPS I often requires weekly infusions of a recombinant form of the enzyme alpha-L-iduronidase (IDUA) which is missing, or defective, in patients with the disorder. While the infusions take several hours, circulating levels of IDUA are undetectable within hours of the treatment due to the replacement protein's short half-life.

"MPS I, including Hurler syndrome, is tremendously debilitating - and actually life-threatening for affected individuals who struggle with progressive disease even using current therapies," said Chester Whitley, Ph.D., M.D., director of the Gene Therapy Center at the University of Minnesota Medical School and the principal investigator on the proposed SB-318-1502 study. "Our studies in mice predict that this innovative treatment will enable the patient's liver to synthesize stable levels of therapeutic enzyme in the circulation, with the goal of significantly impacting disease symptoms and increasing quality life for patients and their families. The goal of this investigation is to more adequately address the terrible and progressive problems of this condition. Searching for innovative treatments is the center of the academic medicine we practice at the University of Minnesota and we appreciate partnerships, like this one with Sangamo, for allowing us to develop and coordinate this type of clinical trial."

"Our proprietary IVPRP genome editing approach allows us to precisely target and edit the albumin 'safe harbor' locus in the DNA of liver cells, with a single administration, which we expect to result in the durable expression of therapeutic enzyme that would be maintained throughout the patient's life," said Geoff Nichol, M.B., Ch.B., Sangamo's executive vice president of research and development. "We believe this approach provides significant advantages over the current standard of care, as well as conventional AAV gene therapy approaches which are non-integrating and have the potential to "wash out" over time as the patient's liver cells divide and turn over. Ultimately, our target population for this approach will be pediatric patients with MPS I, who will benefit most from a one-time, permanent treatment."

"Genome editing has the potential to change the way medicine is practiced, and we have demonstrated that our zinc finger nuclease technology leads the field in the development of therapeutics for both *in vivo* and *ex vivo* applications," said Edward Lanphier, Sangamo's president and chief executive officer. "We are very pleased with the FDA's prompt assessment of our data and their decision to clear our clinical protocol to evaluate our IVPRP approach for the treatment of MPS I. Our goal is to initiate the SB-318-1502 clinical study in mid-2016."

Sangamo remains on track to file additional IND applications for programs employing the IVPRP approach, including MPS II (Hunter syndrome) in the first half of 2016, and hemophilia A, Gaucher disease and Fabry disease in the second half of 2016.

Source:
Sangamo BioSciences, Inc.
