Sangamo Therapeutics Logo

Sangamo Announces FDA Acceptance of IND Application for ST-920 Gene Therapy Candidate for Fabry Disease

February 20, 2019

Company plans to initiate the Phase 1/2 clinical trial for ST-920 in 2019

BRISBANE, Calif., Feb. 20, 2019 /PRNewswire/ -- Sangamo Therapeutics, Inc. (NASDAQ: SGMO), a genomic medicine company, announced today that the U.S. Food and Drug Administration (FDA) has accepted the Investigational New Drug (IND) application for ST-920, a gene therapy candidate being evaluated for the treatment of adults with Fabry disease. Current standard of care for this rare, progressive condition involves regular lifelong infusions of enzyme replacement therapy (ERT).

"The FDA's acceptance of the IND to evaluate ST-920 in Fabry disease enables the initiation of our third clinical development program focused on rare metabolic diseases, and our sixth active clinical program," said Edward Conner, M.D., Chief Medical Officer at Sangamo. "We are eager to commence trials of ST-920 to evaluate our hypothesis that the one-time administration of a gene therapy can fundamentally change the clinical course for patients with Fabry disease."

Fabry disease is an inherited metabolic disease caused by mutations in the *GLA* gene, which result in a deficiency of the enzyme alpha-galactosidase A (α-Gal A). In the absence of functional enzyme, fatty compounds called ganglioside globotriaosylceramides (Gb3) accumulate and can cause serious harm to the skin, kidneys, heart, and the nervous system.

ST-920 comprises an AAV vector carrying a *GLA* gene construct driven by a proprietary liver-specific promoter. ST-920 gene therapy is designed to enable a patient's liver to produce a long-lasting and continuous supply of the α-Gal A enzyme.

The active IND enables Sangamo to initiate a Phase 1/2 clinical trial designed to assess the safety, tolerability and efficacy of ST-920 in adults with Fabry disease. Sangamo expects to open several clinical sites later this year.

About Sangamo Therapeutics

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic medicines with the potential to transform patients' lives using the Company's platform technologies in genome editing, gene therapy, gene regulation and cell therapy. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, Sangamo's plans to initiate the Phase 1/2 clinical trial for ST-920 in 2019; the potential for these trials to evaluate our hypothesis that the one-time administration of a gene therapy can fundamentally change the clinical course for these patients; the design of ST-920 gene therapy to enable a patient's liver to produce a long-lasting and continuous supply of the α-Gal A enzyme; and Sangamo's expectations to open several clinical sites later this year. These statements are not guarantees of future performance and are subject to certain risks, uncertainties and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, the dependence on the success of clinical trials of programs, the lengthy and uncertain regulatory approval process, uncertainties related to the initiation, enrollment and completion of clinical trials, Sangamo's reliance on partners and other third-parties to meet their clinical and manufacturing obligations, and the ability to maintain strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals will be obtained or that Sangamo and its partners will be able to develop commercially viable product candidates. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations and business environments. These risks and uncertainties are described more fully in Sangamo's Quarterly Report on Form 10-Q for the quarter ended September 30, 2018 as filed with the Securities and Exchange Commission. Forward-looking statements contained in this announcement are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

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