Sangamo Announces EMA Releases Details Supporting Orphan Designation for BIVV003 for the Treatment of Sickle Cell Disease

March 17, 2021

BRISBANE, Calif.--(BUSINESS WIRE)--Mar. 17, 2021-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that the European Medicines Agency's Committee for Orphan Medicinal Products (COMP) released details supporting the Orphan Designation of BIVV003, an investigational *ex vivo* gene-edited cell therapy product candidate currently being evaluated for the treatment of sickle cell disease in the Phase 1/2 PRECIZN-1 study partnered with Sanofi. The Committee's decision to grant Orphan Designation was based in part on early data from three patients that had 52 weeks, 13 weeks, and 29 days of follow-up, respectively.

In recently published minutes, the Committee considered the preliminary clinical observations of BIVV003 as well as the potential of long-term effects that may obviate the need for frequent treatment suggested a clinically relevant advantage. The Committee's published minutes report information on select patient characteristics for the first three patients treated, including genotype and history of red blood cell transfusions and vaso-occlusive crises. Sangamo and Sanofi expect to enroll a total of eight patients in the PRECIZN-1 study.

As previously indicated, Sangamo and Sanofi expect to submit updated data from the PRECIZN-1 study for presentation at a medical meeting later this year. At that time, the Companies will also provide an update on the partnered ongoing Thales study evaluating ST-400 in beta thalassemia.

About Sangamo Therapeutics

Sangamo Therapeutics is committed to translating ground-breaking science into genomic medicines with the potential to transform patients' lives using gene therapy, cell therapy, and genome engineering. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements

This press release contains forward-looking statements regarding our current expectations. These forward-looking statements include, without limitation, statements relating to the therapeutic potential of BIVV003, including its potential clinical benefit to patients with sickle cell disease and as an alternative to the standard of care for patients with sickle cell disease, the anticipated plans and timelines of Sangamo and our collaborators for enrolling and treating patients in and conducting the PRECIZN-1 clinical study and presenting clinical data from the PRECIZN-1 and Thales clinical studies and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to the effects of the evolving COVID-19 pandemic and the impacts of the pandemic on the global business environment, healthcare systems and business and operations of Sangamo and our collaborators, including the operation of clinical trials; the research and development process; the uncertain timing and unpredictable nature of clinical trial results, including the risks that therapeutic effects observed in clinical trial results will not be durable in patients and that final clinical trial data will not validate the safety and efficacy of BIVV003; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; the manufacturing of products and product candidates; the commercialization of approved products; the potential for technological developments that obviate technologies used by Sangamo and Sanofi; the potential for Sanofi to terminate the BIVV003 or ST-400 programs or to breach or terminate its collaboration agreements with Sangamo; and the potential for Sangamo to fail to realize its expected benefits of its collaborations with Sanofi, including the risk that Sangamo may not earn any additional milestone or royalty payments under its collaborations with Sanofi.

There can be no assurance that we and our collaborators will be able to develop commercially viable products. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in the operations and business environments of Sangamo and our collaborators. These risks and uncertainties are described more fully in our Securities and Exchange Commission filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2020. Forward-looking statements contained in this announcement are made as of this date, and we undertake no duty to update such information except as required under applicable law.

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