



## Sangamo Therapeutics Announces Updated Preliminary SAR445136 Phase 1/2 Proof-of-Concept Data Showing Tolerability and Sustained Effects in Sickle Cell Disease

December 12, 2021

- No adverse events related to investigational SAR445136 were reported
- All four treated patients experienced increases in total hemoglobin, fetal hemoglobin and percent F cells; none required blood transfusions post engraftment

BRISBANE, Calif.--(BUSINESS WIRE)--Dec. 12, 2021-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced updated preliminary proof-of-concept clinical data from the Phase 1/2 PRECIZN-1 study of SAR445136, a zinc finger nuclease gene-edited cell therapy candidate in development with Sanofi for the treatment of sickle cell disease (SCD). These data are being presented today at the 63<sup>rd</sup> American Society for Hematology Annual Meeting and Exposition taking place from December 11-14 virtually and in Atlanta, GA. The poster presentation, which includes follow-up data up to 91 weeks for the longest-treated patient, is available on Sangamo's website in the Investors and Media section under [Events and Presentations](#).

"We are very pleased with these updated preliminary proof-of-concept efficacy and safety results, which we believe illustrate the therapeutic potential of zinc finger nuclease engineered cell therapy to address the current unmet needs of patients with sickle cell disease," said Rob Schott, M.D., M.P.H., F.A.C.C, Head of Development at Sangamo. "We believe this is an important demonstration of our versatile zinc finger platform translating meaningfully into the clinic."

As of the September 22, 2021 cutoff date, the most recently treated patient in the PRECIZN-1 Phase 1/2 study had been followed for 26 weeks and the longest-treated patient had been followed for 91 weeks. None of the four treated patients required blood transfusions post engraftment. Total hemoglobin stabilized by Week 26 after treatment with SAR445136 in all four patients. Fetal hemoglobin level increased from 0.1-11% at screening to 14-39% by Week 26 in all four patients and was 38% in the longest-treated patient at 91 weeks. Percent F cells increased to 64-96% by 39 weeks of follow-up in all four patients, persisting at 99% in the patient with 91 weeks of follow-up. The SAR445136 investigational drug product had on-target *BCL11A* gene modification (61-78%) in all four patients.

"These preliminary proof of concept efficacy and safety results support the potential therapeutic value of the zinc finger nuclease-mediated modification of the *BCL11E* region," said Karin Knobe, Head of Development, Rare Diseases and Rare Blood Disorders at Sanofi.

As of the cutoff date, there were no adverse events (AEs) assessed as related to SAR445136. Most AEs reported in the screening, mobilization, apheresis and conditioning periods were SCD-related events. One serious adverse event of sickle cell anemia with crisis (vaso-occlusive crisis or VOC) was reported approximately nine months after treatment with SAR445136 in one patient, and no other SCD-related events were reported in the four patients post-infusion.

Additional data from this study are expected to be presented at a medical meeting in 2022.

### About the PRECIZN-1 study

PRECIZN-1 is an ongoing first-in-human, open label, single arm, multi-site Phase 1/2 study in up to eight patients with SCD evaluating the safety and tolerability of cell therapy candidate SAR445136. The therapeutic product is manufactured by *ex vivo* gene editing of a patient's own (autologous) hematopoietic stem cells using non-viral delivery of zinc finger nuclease technology targeting the *BCL11a* gene erythroid-specific enhancer (ESE) to increase endogenous fetal hemoglobin (HbF) production. SAR445136 has received Fast Track Designation from the FDA and Orphan Medicinal Product from the EMA. The safety and efficacy of SAR445136 has not been reviewed by any regulatory authority worldwide.

### About Sangamo Therapeutics

Sangamo Therapeutics is a clinical-stage biopharmaceutical company with a robust genomic medicines pipeline. Using ground-breaking science, including our proprietary zinc finger genome engineering technology and manufacturing expertise, Sangamo aims to create new genomic medicines for patients suffering from diseases for which existing treatment options are inadequate or currently don't exist. For more information about Sangamo, visit [www.sangamo.com](http://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, statements regarding the therapeutic potential of SAR445136, including its potential clinical benefit to patients with sickle cell disease and its potential as an alternative to the standard of care for patients with sickle cell disease, 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. Sangamo's actual results may differ materially and adversely from those expressed in these forward looking statements. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the evolving COVID-19 pandemic and its impact on the global business environment, healthcare systems and the business and operations of Sangamo and Sanofi, including the initiation and 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 the preliminary proof-of-concept results from the Phase 1/2 PRECIZN-1 study will not be durable in patients and that final clinical trial data will not validate the safety and efficacy of SAR445136; reliance on results of early clinical trials, which results are not necessarily predictive of future clinical trial results; 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 in SAR445136; the potential for Sanofi to terminate the SAR445136 program or to breach or terminate its collaboration*

*agreement with Sangamo; and the potential for Sangamo to fail to realize its expected benefits of its collaboration with Sanofi, including the risk that Sangamo may not earn any additional milestone or royalty payments under its collaboration with Sanofi. These risks and uncertainties are described more fully in Sangamo's filings with the U.S. Securities and Exchange Commission, including its Annual Report on Form 10-K for the year ended December 31, 2020 and the most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2021. The information contained in this release is as of December 12, 2021, and Sangamo undertakes no duty to update forward-looking statements contained in this release except as required by applicable laws.*

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