Sangamo Therapeutics Logo

Sangamo Highlights Advancements in Genomic Medicine Pipeline and Expanded R&D and Manufacturing Capabilities at R&D Day

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- IND transfer to Pfizer for SB-525 hemophilia A gene therapy is substantially completed; Pfizer is advancing SB-525 into a Phase 3 registrational study in 2020
- At R&D Day, Sangamo is detailing global capabilities across clinical science, operations, product development, and manufacturing
- Company is also introducing new gene therapy and genome regulation programs for clinical development, including several addressing highly prevalent diseases, with IND targets in 2021 and 2022
- Live webcast today at 8am Eastern Time

BRISBANE, Calif.--(BUSINESS WIRE)--Dec. 17, 2019-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, is hosting an R&D Day today beginning at 8am Eastern Time. During the event, Sangamo executives and scientists plan to provide updates across the Company's clinical and preclinical pipeline, as well as an overview of manufacturing capabilities to support clinical and commercial supply. A live webcast link will be available on the Events and Presentations page of the Sangamo website

"The talent, R&D capabilities, manufacturing expertise, and operations infrastructure we have brought to Sangamo have enabled us to advance a genomic medicine pipeline that spans multiple therapeutic areas and now also extends into late-stage development," said Sandy Macrae, CEO of Sangamo. "As we make progress in clinical development, we gain insights into the use of our technology and are applying those insights as we advance new programs, such as the gene therapy for PKU and the genome regulation candidates for CNS diseases we are announcing today."

Macrae continued: "We will continue to pursue a dual approach of retaining certain programs for our proprietary pipeline while also establishing pharmaceutical partnerships to gain access to therapeutic area expertise and financial, operational, and commercial resources. Strategic collaborations will be a particularly important consideration as we advance programs for diseases affecting large patient populations."

R&D Day updates on clinical and preclinical pipeline programs:

Gene therapy product candidates for hemophilia A, Fabry disease, and PKU

SB-525 is a gene therapy product candidate for hemophilia A being developed by Sangamo and Pfizer under a global development and commercialization collaboration agreement. The transfer of the SB-525 IND to Pfizer is substantially completed. Pfizer is advancing SB-525 into a Phase 3 registrational study in 2020 and has recently begun enrolling patients into a Phase 3 lead-in study.

At R&D Day, Sangamo executives are presenting data from the SB-525 program which were recently announced at the American Society of Hematology (ASH) annual meeting.

The cassette engineering, AAV engineering and manufacturing expertise which Sangamo used in the development of SB-525 are also being applied to the ST-920 Fabry disease program, which is being evaluated in a Phase 1/2 clinical trial, as well as to the newly announced ST-101 gene therapy program for PKU, which is being evaluated in preclinical studies with a planned IND submission in 2021.

Engineered ex vivo cell therapy candidates for beta thalassemia, kidney transplantation, and preclinical research in multiple sclerosis (MS)

Sangamo is providing an overview of the Company's diversified cell therapy pipeline this morning. Cell therapy incorporates Sangamo's experience and core strengths, including cell culture and engineering, gene editing, and AAV manufacturing. At R&D Day, Sangamo scientists today are reviewing the early data presented this month at ASH from the ST-400 beta thalassemia *ex vivo* gene-edited cell therapy program, which is being developed in partnership with Sanofi.

Sangamo is also providing updates on the company's CAR- $_{TREG}$ clinical and preclinical programs. CAR-$_{TREGS}$ are regulatory T cells equipped with a chimeric antigen receptor. Sangamo is the pioneer in CAR-$_{TREGS}$, which may have the potential to treat inflammatory and autoimmune diseases. TX200 is being evaluated in the **ST**EADFAST study, the first ever clinical trial evaluating a CAR-$_{TREG}$ cell therapy. Tx200 is being developed for the prevention of immune-mediated organ rejection in patients who have received a kidney transplant, a significant unmet medical need. Results from this trial will provide data on safety and proof of mechanism, building a critical understanding of CAR-$_{TREGS}$ in patients, and may provide a gateway to autoimmune indications such as Crohn's disease and multiple sclerosis (MS). Sangamo is also presenting preclinical murine data demonstrating that CAR-$_{TREGS}$ accumulate and proliferate in the CNS and reduce a marker of MS.

In vivo genome editing optimization

Clinical data presented earlier this year provided evidence that Sangamo had successfully edited the genome of patients with mucopolysaccharidosis type II (MPS II) but also suggested that the zinc finger nuclease *in vivo* gene editing reagents were under-dosed using first-generation technology. Sangamo has identified potential improvements that may enhance the potency of *in vivo* genome editing, including increasing total AAV vector dose, co-packaging both ZFNs in one AAV vector, and engineering second-generation AAVs, ZFNs, and donor transgenes.

Genome regulation pipeline candidates targeting neurodegenerative diseases including Alzheimer's and Parkinson's

Sangamo scientists today are presenting data demonstrating that the company's engineered zinc finger protein transcription factors (ZFP-TFs)

specifically and powerfully repress key genes involved in brain diseases including Alzheimer's, Parkinson's, Huntington's, ALS, and Prion diseases. Sangamo is advancing its first two genome regulation programs toward clinical development:

- ST-501 for tauopathies including Alzheimer's, with an IND anticipated in 2021
- ST-502 for alpha-synuclein diseases including Parkinson's, with an IND anticipated in 2022

Sangamo scientists are also presenting data demonstrating progress in the development of new AAV serotypes for use in CNS diseases.

Manufacturing capabilities and strategy

Sangamo is nearing completion of its buildout of a GMP manufacturing facility at the new Company headquarters in Brisbane, CA. This facility is expected to become operational in 2020 and to provide clinical and commercial scale manufacturing capacity for cell and gene therapy programs. The Company has also initiated the buildout of a cell therapy manufacturing facility in Valbonne, France. Sangamo's manufacturing strategy includes in-house capabilities as well as the use of contract manufacturing organizations, including a long-established relationship with Thermo Fisher Scientific for clinical and large-scale commercial AAV manufacturing capacity.

R&D Day webcast

A live webcast of the R&D Day, including audio and slides, will be available on the Events and Presentations page of the Sangamo website today at 8am Eastern Time. A replay of the event will be archived on the website.

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, *ex vivo* gene-edited cell therapy, and *in vivo* genome editing and gene regulation. For more information about Sangamo, visit www.sangamo.com.

Sangamo Forward Looking Statements

This press release contains forward-looking statements within the meaning of the "safe harbor" provisions of United States securities law. These forward-looking statements include, but are not limited to, the therapeutic potential of Sangamo's product candidates; the design of clinical trials and expected timing for milestones, such as enrollment and presentation of data, the expected timing of release of additional data, plans to initiate additional studies for product candidates and timing and design of these studies; the expected benefits of Sangamo's collaborations; the anticipated capabilities of Sangamo's technologies; the research and development of novel gene-based therapies and the application of Sangamo's ZFP technology platform to specific human diseases; successful manufacturing of Sangamo's product candidates; the potential of Sangamo's genome editing technology to safely treat genetic diseases; the potential for ZFNs to be effectively designed to treat diseases through genome editing; the potential for cell therapies to effectively treat diseases; and other statements that are not historical fact. These statements are based upon Sangamo's current expectations and speak only as of the date hereof. Sangamo's actual results may differ materially and adversely from those expressed in any forward-looking statements. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to dependence on the success of clinical trials; the uncertain regulatory approval process; the costly research and development process, including the uncertain timing of clinical trials; whether interim, preliminary or initial data from ongoing clinical trials will be representative of the final results from such clinical trials; whether the final results from ongoing clinical trials will validate and support the safety and efficacy of product candidates; the risk that clinical trial data are subject to differing interpretations by regulatory authorities; Sangamo's limited experience in conducting later stage clinical trials and the potential inability of Sangamo and its partners to advance product candidates into registrational studies; Sangamo's reliance on itself, partners and other third-parties to meet clinical and manufacturing obligations; Sangamo's ability to maintain strategic partnerships; competing drugs and product candidates that may be superior to Sangamo's product candidates; and the potential for technological developments by Sangamo's competitors that will obviate Sangamo's gene therapy technology. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations. This presentation concerns investigational drugs that are under preclinical and/or clinical investigation and which have not yet been approved for marketing by any regulatory agency. They are currently limited to investigational use, and no representations are made as to their safety or effectiveness for the purposes for which they are being investigated. Any discussions of safety or efficacy are only in reference to the specific results presented here and may not be indicative of an ultimate finding of safety or efficacy by regulatory agencies. These risks and uncertainties are described more fully in Sangamo's Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the Securities and Exchange Commission on March 1, 2019 and Sangamo's Quarterly Report on Form 10-Q for the quarter ended September 30, 2019 that it filed on or about November 6, 2019. Except as required by law, we assume no obligation, and we disclaim any intent, to update these statements to reflect actual results.

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Investor Relations – Global
McDavid Stilwell
510-970-6000, x219
mstilwell@sangamo.com