



# Sangamo and Pfizer announce collaboration for development of zinc finger protein gene therapy for ALS

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Sangamo Therapeutics, Inc. (Nasdaq: SGMO) and Pfizer Inc. (NYSE: PFE) today announced a collaboration for the development of a potential gene therapy using zinc finger protein transcription factors (ZFP-TFs) to treat amyotrophic lateral sclerosis (ALS) and frontotemporal lobar degeneration (FTLD) linked to mutations of the C9ORF72 gene.

ALS and FTLD are part of a spectrum of neurodegenerative disorders caused by mutations in the C9ORF72 gene that involves hundreds of additional repetitions of a six base pair sequence of DNA. This ultimately leads to the deterioration of motor neurons, in the case of ALS, or neurons in the frontal and temporal lobes, in the case of FTLD. Currently, there are no cures to halt or reverse the progression of ALS or FTLD. The C9ORF72 mutation is linked to approximately one-third of cases of familial ALS.

“We are excited to continue our collaborative relationship with Pfizer with this new program using Sangamo’s zinc finger protein technology to develop a potential gene therapy for patients with certain forms of ALS and FTLD, devastating diseases with very limited treatment options,” said Dr. Sandy Macrae, Chief Executive Officer of Sangamo. “The precision and flexibility of zinc finger proteins enables targeting of virtually any genetic mutation. Collaboration with the right partner for a given therapeutic application is a key component of our corporate strategy and enables us to pursue the vast opportunity set of our platform.”

"We look forward to working with Sangamo on potential treatments for devastating diseases related to genetic mutations of the C9ORF72 gene," said Greg LaRosa, Senior

Vice President and Chief Scientific Officer, Pfizer Rare Disease. "Pfizer is proud of the progress we have made in the area of gene therapy, which offers tremendous promise to patients and their families."

Gene therapies are a potentially transformational technology for patients, focused on highly specialized, one-time treatments that address the root cause of diseases caused by genetic mutation. Sangamo's ZFP-TF technology involves introducing an engineered zinc finger protein (ZFP) which is designed to identify and bind to a precise sequence of DNA. Once bound to the target sequence of DNA, a transcriptional repressor domain attached to the ZFP suppresses expression of the gene. Under this collaboration, Sangamo and Pfizer will investigate allele-specific ZFP-TFs with the potential to differentiate the mutant C9ORF72 allele from the wild type allele and to specifically down-regulate expression of the mutant form of the gene.

Under the terms of the collaboration agreement, Sangamo will receive a \$12 million upfront payment from Pfizer. Sangamo will be responsible for the development of ZFP-TF candidates. Pfizer will be operationally and financially responsible for subsequent research, development, manufacturing and commercialization for the C9ORF72 ZFP-TF program and any resulting products. Sangamo is eligible to receive potential development and commercial milestone payments of up to \$150 million, as well as tiered royalties on net sales.

In May 2017, Sangamo and Pfizer entered into an exclusive, global collaboration and license agreement for the development and commercialization of potential gene therapy products for Hemophilia A, including SB-525, which entered the clinic in August 2017.

**About Sangamo's ZFP-TF Gene Regulation Platform** Sangamo's zinc finger protein transcription factor (ZFP-TF)-mediated gene regulation approach is designed to either selectively repress (down-regulate) or activate (up-regulate) the expression of a specific gene or DNA sequence with a single administration. This technology enables targeting of a broad range of diseases requiring regulation of endogenous gene expression and differs from other approaches such as gene therapy or zinc finger nuclease-mediated genome editing, which are designed to replace or correct a missing or mutated gene or DNA sequence. Sangamo is developing ZFP-TFs as a novel therapeutic approach for diseases of the central nervous system (CNS). In keeping with the company's strategy to externalize development of ZFP-TFs for CNS diseases, Sangamo has established collaborations with Pfizer for ALS and FTLD and with Shire for Huntington's disease. Sangamo is also developing ZFP-TFs to down-regulate the expression of tau, a protein associated with Alzheimer's disease and frontotemporal dementia (FTD). The company's

strategy for the tau program is to seek a development and commercialization partner upon completion of preclinical studies.

**About Sangamo Therapeutics** Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company is conducting Phase 1/2 clinical trials in Hemophilia A and Hemophilia B, and in lysosomal storage disorders MPS I and MPS II. Sangamo has an exclusive, global collaboration and license agreement with Pfizer Inc. for gene therapy programs for Hemophilia A, ALS and FTLD, with Bioverativ Inc. for hemoglobinopathies, including beta thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, Sangamo has established strategic partnerships with companies in non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at [www.sangamo.com](http://www.sangamo.com).

**About Pfizer Inc.: Working together for a healthier world®** At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at [www.pfizer.com](http://www.pfizer.com). In addition, to learn more, please visit us on [www.pfizer.com](http://www.pfizer.com) and follow us on Twitter at @Pfizer and @Pfizer\_News, LinkedIn, YouTube and like us on Facebook at [Facebook.com/Pfizer](https://www.facebook.com/Pfizer).

**Sangamo Forward Looking Statements** This press release contains forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation references relating to research and development of therapeutic applications of Sangamo's gene therapy and ZFP technology platforms, the potential of Sangamo's ZFP technology to treat ALS and FTLD, the potential success and benefits of Sangamo's corporate strategy to partner with other pharmaceutical

companies, and anticipated milestones and royalties. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to the ability of Sangamo's ZFP-TF technology to treat successfully diseases like ALS and FTLN, the inability to execute on Sangamo's corporate strategy to partner with other pharmaceutical companies or collaborate successfully, the inability to achieve anticipated milestones and the inability to develop commercially viable products. For a more detailed discussion of these and other risks, please see Sangamo's SEC filings, including the risk factors described in its most recent Quarterly Report on Form 10-Q. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

**Pfizer Disclosure Notice:** The information contained in this release is as of January 3, 2018. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments. This release contains forward-looking information about ZFP-TFs, a collaboration for the development of a potential gene therapy using ZFP-TFs for the treatment of ALS and FTLN and the potential of gene therapy, including their potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical study commencement and completion dates as well as the possibility of unfavorable study results, including unfavorable new clinical data and additional analyses of existing clinical data; risks associated with initial data; the risk that clinical trial data are subject to differing interpretations, and, even when we view data as sufficient to support the safety and/or effectiveness of a product candidate, regulatory authorities may not share our views and may require additional data or may deny approval altogether; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when any applications may be filed with regulatory authorities for any potential gene therapies; whether and when regulatory authorities may approve any such applications, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted, and, if approved, whether any such gene therapies will be commercially successful; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of any such gene therapies; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2016 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-

Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at [www.sec.gov](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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