



Sangamo Announces Global Collaboration With Novartis to Develop Genomic Medicines for Autism and Other Neurodevelopmental Disorders

July 30, 2020

- Collaboration for gene regulation therapies for three neurodevelopmental targets, including for genes linked to autism spectrum disorder and intellectual disability

- Sangamo will receive a \$75M upfront licensing fee and is eligible to receive up to \$720M in potential milestones, as well as royalties on potential net commercial sales

BRISBANE, Calif.--(BUSINESS WIRE)--Jul. 30, 2020-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that it has executed a global licensing collaboration agreement with Novartis to develop and commercialize gene regulation therapies to address three neurodevelopmental targets, including autism spectrum disorder (ASD) and other neurodevelopmental disorders. The collaboration will leverage Sangamo's proprietary genome regulation technology, zinc finger protein transcription factors (ZFP-TFs), to aim to upregulate the expression of key genes involved in neurodevelopmental disorders.

"At Sangamo, we believe that we can engineer zinc finger proteins to address virtually any genomic target, and we are building a broad pipeline of wholly owned and partnered programs with the goal to bring our genomic medicines to patients. In the case of the central nervous system, there are potentially hundreds of neurological disease gene targets that may be addressable by our zinc finger platform," said Sandy Macrae, CEO of Sangamo. "Partnering Sangamo's proprietary technology with Novartis' deep experience in neuroscience drug development is a powerful combination which expands Sangamo's pipeline and allows us to tackle challenging neurodevelopmental conditions. Our goal in this collaboration is to create genomic medicines for patients with neurodevelopmental disorders, such as autism, that can potentially alter the natural history of these complex lifelong disorders."

"This collaboration with Sangamo is part of our commitment to pioneering the next generation of neurodevelopmental treatments," said Jay Bradner, President of the Novartis Institutes for BioMedical Research. "The goal is to create new gene regulation therapies that act at the genomic level, moving us beyond the symptom focused treatments of today and toward therapies that can address some of the most challenging neurodevelopmental disorders."

Sangamo's ZFP-TF genome regulation technology, which is currently delivered with adeno-associated viruses (AAVs), functions at the DNA level to selectively repress or activate the expression of specific genes to achieve a desired therapeutic effect. The collaboration will leverage ZFP-TFs engineered by Sangamo scientists in an effort to upregulate, or activate, the expression of genes that are inadequately expressed in individuals with certain types of neurodevelopmental disorders.

Under the terms of the agreement, over a three-year collaboration period, Novartis has exclusive rights to ZFP-TFs targeted to three undisclosed genes which are associated with neurodevelopmental disorders, including ASD and intellectual disability. Novartis also has the option to license Sangamo's proprietary AAVs. Sangamo is responsible for certain research and associated manufacturing activities, all of which will be funded by Novartis, and Novartis assumes responsibility for additional research activities, investigational new drug-enabling studies, clinical development, related regulatory interactions, manufacturing and global commercialization.

Under the collaboration agreement, Novartis will pay Sangamo a \$75 million upfront license fee payment within thirty days. In addition, Sangamo is eligible to earn up to \$720 million in other development and commercial milestone payments, including up to \$420 million in development milestones and up to \$300 million in commercial milestones. Sangamo is also eligible to receive from Novartis tiered high single-digit to sub-teen double-digit royalties on potential net commercial sales of products arising from the collaboration.

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.

Forward Looking Statements

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to the potential to develop, obtain regulatory approvals for and commercialize safe and effective therapies to treat neurodevelopmental disorders and the timing and availability of such therapies, the potential for Sangamo to receive upfront licensing fees and earn milestone payments and royalties under the Novartis collaboration and the timing of such fees, payments and royalties, the potential to use ZFP-TF technology to upregulate specific genes involved with neurodevelopmental disorders, the therapeutic potential of Sangamo's zinc finger platform and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to 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 evolving COVID-19 pandemic and its impact on the global business environment, healthcare systems and the business and operations of Sangamo and Novartis; the research and development process; 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 Novartis; the potential for Novartis to breach or terminate the collaboration agreement; and the potential for Sangamo to fail to realize its expected benefits of the Novartis collaboration. There can be no assurance that Sangamo will earn any milestone or royalty payments under the Novartis collaboration or obtain regulatory approvals for product candidates arising from these collaborations. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's and

Novartis' operations and business environments. These risks and uncertainties are described more fully in Sangamo's filings with the U.S. Securities and Exchange Commission, including its most recent Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 and Annual Report on Form 10-K for the year ended December 31, 2019. 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.

View source version on [businesswire.com](https://www.businesswire.com/news/home/20200730005166/en/): <https://www.businesswire.com/news/home/20200730005166/en/>

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

Media Inquiries – Global
Aron Feingold
510-970-6000, x421
afeingold@sangamo.com

Investor Relations and Media Inquiries – European Union & United Kingdom
Caroline Courme
33 4 97 21 27 27
ccourme@sangamo.com

Source: Sangamo Therapeutics, Inc.