

# Global licensing collaboration between Sangamo and Novartis to develop genomic medicines for three neurodevelopmental targets.

## Sangamo and Novartis Collaboration



- Strategically partners Sangamo's proprietary genome regulation technology with Novartis' deep experience pioneering treatments in neurodevelopment.
- Leverages Sangamo's zinc finger protein transcription factors (ZFP-TFs) to upregulate, or activate, expression of genes that are inadequately expressed with certain neurodevelopmental disorders.
- Aims to develop and commercialize gene regulation therapies to address three neurodevelopmental targets, including genes linked to autism spectrum disorder (ASD) and other neurodevelopmental targets.

## Scope and responsibilities



- Over a three-year period, Novartis has exclusive rights to ZFP-TFs targeted to three undisclosed genes associated with neurodevelopmental disorders, including ASD and intellectual disability.
- Novartis 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.
- Novartis will assume responsibility for additional research activities, IND-enabling studies, clinical development, related regulatory interactions, manufacturing, and global commercialization.

## By the numbers

**\$75M**

Upfront license fee

**\$720M**

Potential milestone payments include \$420M in development milestones and \$300M in commercial milestones

**Net sales**  
**%**

Royalties in the high single to sub-teen double digits

"Partnering Sangamo's proprietary technology with Novartis' deep experience in neuroscience drug development is a powerful combination which will expand Sangamo's pipeline and allow 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."

**Sandy Macrae**

Sangamo's Chief Executive Officer

"This collaboration with Sangamo is part of our commitment to pioneering the next generation of neurodevelopmental treatments. The goal is to create new gene control 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."

**Jay Bradner**

President, Novartis Institutes for BioMedical Research