



Sangamo Therapeutics and Voyager Therapeutics Enter License Agreement for Epigenetic Regulation Treatment of Prion Disease

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BRISBANE, Calif. & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, and Voyager Therapeutics, Inc. (Nasdaq: VYGR), today announced the parties have entered into a definitive license agreement for a potential treatment of prion disease. Using its proprietary epigenetic regulation platform, Sangamo has developed zinc finger transcriptional regulators (ZF-TRs) which it believes can specifically and potentially block expression of the prion protein, the pathogenic driver of prion disease. Sangamo's ZF-TRs have been shown in animal models to significantly reduce expression of the prion protein in the brain, extend life span and limit formation of toxic prion aggregates. Under the agreement, Voyager has provided Sangamo with access to a capsid derived from Voyager's proprietary TRACER™ platform. This capsid has been shown to effectively cross the blood-brain barrier when delivered intravenously in animal models.

"Prion is a devastating, rapidly progressing and deadly disease, for which no treatment options currently exist," said Sandy Macrae, Chief Executive Officer at Sangamo. "While evaluating delivery mechanisms for our zinc finger transcriptional regulators, we were pleased to identify Voyager's capsid as being well suited to potentially achieve the specific central nervous system coverage required for this indication today. Our agreement with Voyager highlights the importance of collaboration to address difficult indications with significant unmet medical need. I'm excited to combine Sangamo's cutting-edge epigenetic regulation capabilities with the delivery abilities of Voyager's capsid to potentially create the first ever meaningful therapy for prion disease."

Under the terms of the agreement, Sangamo has received a non-exclusive license to combine a Voyager TRACER capsid with Sangamo's ZF-TRs designed to treat prion disease. Sangamo is solely responsible for the research, development, manufacture and commercialization of any product candidates using the Voyager capsid. Voyager is eligible to earn certain license fees, royalties on potential commercial sales of any products using Voyager's capsid, and, in the event the prion program is out licensed by Sangamo, a portion of all licensing revenues received with respect to this program. Sangamo expects to submit a potential IND for a product candidate treating prion disease in 2025.

"We believe the novel, brain-penetrant capsids emerging from our TRACER capsid discovery platform have the potential to enable gene therapies for a wide variety of diseases of the central nervous system – far more than we could prosecute internally," said Alfred W. Sandroock, Jr., M.D., Ph.D., Chief Executive Officer of Voyager. "We continue to explore a variety of capsid license structures, such as in this transaction with Sangamo, to leverage our technology for more programs, and ultimately, more patients."

About Prion Disease

Prion disease is a rapidly progressing, fatal neurodegenerative disease caused by the misfolding of the cellular prion protein, PrP^C, encoded by the *PRNP* gene. Misfolded prion protein is acutely toxic to neurons, which can lead to the rapid development of dementia, difficulty walking and changes in gait, hallucinations, muscle stiffness, confusion, fatigue, difficulty speaking, and ultimately death. Most cases are sporadic or caused by inherited dominant mutations in *PRNP*, with an estimated 500 patients diagnosed per year in the US. The most common form of prion disease that affects humans is Creutzfeldt-Jakob disease. There are currently no approved or clinical-stage disease-modifying therapies for the prevention or treatment of prion disease.

About Sangamo Zinc Finger Transcriptional Regulators

Sangamo's zinc finger transcriptional regulators, or ZF-TRs, recognize and bind to a specific DNA sequence within or near a particular gene, allowing expression of that target gene to be potentially regulated. ZF activators, or ZF-As, are created by attaching a zinc finger array to an activation domain with the aim of increasing the expression of a target gene relative to an untreated cell. ZF repressors, or ZF-Rs, are created by attaching a zinc finger array to a repression domain in order to down regulate or completely turn off a gene. ZF-Rs can also be designed to selectively repress expression of a mutant allele while allowing for the expression of the healthy allele. Sangamo is currently evaluating ZF-TRs in a variety of pre-clinical programs, including Nav1.7 for the potential treatment of chronic neuropathic pain and for prion disease.

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. To learn more, visit www.sangamo.com and connect with us on [LinkedIn](#) and [Twitter](#).

About the TRACER™ AAV Capsid Discovery Platform

Voyager's TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) capsid discovery platform is a broadly applicable, RNA-based screening platform that enables rapid discovery of AAV capsids with robust penetration of the blood-brain barrier and enhanced central nervous system (CNS) tropism in multiple species, including non-human primates (NHPs). TRACER generated capsids have demonstrated superior and widespread gene expression in the CNS compared to conventional AAV capsids as well as cell- and tissue-specific transduction, including to areas of the brain that have been traditionally difficult to reach. Separate results have demonstrated the enhanced ability of certain capsids to target cardiac muscle and to de-target the dorsal root ganglia. Voyager is expanding its library of AAV capsids optimized to deliver diverse therapeutic payloads to address a broad range of CNS and other diseases. As part of its external partnership strategy, Voyager has established multiple collaboration agreements providing access to its next-generation TRACER capsids to potentially enable its partners' gene therapy programs to treat a variety of diseases.

About Voyager Therapeutics

Voyager Therapeutics (Nasdaq: VYGR) is a biotechnology company dedicated to breaking through barriers in gene therapy and neurology. The potential of both disciplines has been constrained by delivery challenges; Voyager is leveraging cutting-edge expertise in capsid discovery and deep neuropharmacology capabilities to address these constraints. Voyager's TRACER™ AAV capsid discovery platform has generated novel capsids with high target delivery and blood-brain barrier penetration at low doses, potentially addressing the narrow therapeutic window associated with conventional gene therapy delivery vectors. This platform is fueling alliances with Pfizer Inc., Novartis and Neurocrine Biosciences as well as multiple programs in Voyager's own pipeline. Voyager's pipeline includes wholly owned and collaborative preclinical programs in Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Parkinson's disease, and Friedreich's Ataxia, with a focus on validated targets and biomarkers to enable a path to rapid potential proof-of-biology. For more information, visit www.voyagertherapeutics.com.

Voyager Therapeutics® is a registered trademark, and TRACER™ is a trademark, of Voyager Therapeutics, Inc.

Sangamo Forward Looking Statements

This press release contains forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to zinc finger transcriptional regulators' potential to specifically and potently block expression of the prion protein, the development of a potential treatment for prion disease through epigenetic regulation of the prion protein and the occurrence and timing of a potential IND submission in 2025. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, the research and development process, including the results of clinical trials; the regulatory approval process for product candidates; and the potential for technological developments that obviate technologies used by Sangamo and Voyager; the COVID-19 pandemic; the potential for Voyager to breach or terminate its agreement with Sangamo; and the potential for Sangamo to fail to realize its expected benefits of the Voyager agreement. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations and business. These risks and uncertainties are described more fully in our Securities and Exchange Commission filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2022, and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023. 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.

Voyager Forward Looking Statements

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "might," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "undoubtedly," "target," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward-looking statements.

For example, all statements Voyager makes regarding Voyager's ability to continue to develop its TRACER capsid discovery platform and identify and develop proprietary, brain-penetrant capsids from its TRACER capsid discovery platform with the potential to enable gene therapies for a wide variety of diseases of the central nervous system; Voyager's ability to utilize its proprietary capsids in its own product development programs; Voyager's ability to attract parties to license its novel proprietary capsids or to participate with Voyager in research and development collaborations utilizing its novel proprietary capsids; Voyager's ability to advance its AAV-based gene therapy programs; Voyager's entitlement to receive license fees and royalties from Sangamo under the license agreement, Voyager's ability to add new programs to its pipeline and enter into new partnerships or collaborations, and the sufficiency of Voyager's cash resources to enable it to continue to identify and develop proprietary capsids from its TRACER capsid discovery platform are forward-looking.

All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Voyager expected. Such risks and uncertainties include, among others, the continued development of Voyager's capsid and gene therapy platforms; Voyager's ability to attract and retain talented contractors and employees to continue the development of the TRACER capsid discovery platform and the identification of proprietary capsids; Voyager's ability to create and protect intellectual property rights associated with the TRACER capsid discovery platform and the capsids identified by the platform; the possibility or the timing of any development or commercialization under the Sangamo license agreement and other license and collaboration agreements; the ability of Voyager to negotiate and complete licensing or collaboration agreements on terms acceptable to Voyager and third parties; Voyager's ability to perform its obligations under its license option agreement with Sangamo's ability to perform its obligations under such agreement; and the sufficiency of Voyager's cash resources to enable it to fund its operations and development plans. These statements are also subject to a number of material risks and uncertainties that are described in Voyager's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, as updated by its subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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