



Ocugen and CanSinoBIO Enter Strategic Partnership for Gene Therapy Co-Development and Manufacturing

September 30, 2019

Partnership to develop OCU400, Ocugen's orphan drug designated gene therapy candidate in its modifier gene therapy platform

MALVERN, Pa. & TIANJIN, China--(BUSINESS WIRE)--Sep. 30, 2019-- [Ocugen, Inc.](#), (NASDAQ: OCGN), a clinical stage biopharmaceutical company focused on innovative therapies that address rare and underserved eye diseases, has entered into a strategic partnership with CanSino Biologics ("CanSinoBIO")(6185.HK) on Ocugen's gene therapy pipeline product candidates for inherited retinal diseases, which are currently in development with Schepens Eye Research Institute of Massachusetts Eye and Ear, an affiliate of Harvard Medical School.

Under this strategic collaboration, CanSinoBIO will provide all CMC development and clinical supplies for the development of OCU400, Ocugen's first gene therapy product candidate in its modifier gene therapy platform. CanSinoBIO maintains the option to support commercial manufacturing for Ocugen. The agreement also provides commercialization rights to CanSinoBIO in Greater China.

"We believe our modifier gene therapy platform, and OCU400 as its first product candidate, has the potential to treat many inherited retinal diseases with one product," said Shankar Musunuri, Ph.D., MBA, Chairman, CEO and Co-Founder of Ocugen. "A reliable manufacturing partnership is critical for gene therapy clinical trials and commercialization. Partnership with CanSinoBIO, with their state-of-the-art facilities and world class team, provides us a clear path to advance our development and manufacturing processes to reach the clinic."

OCU400 has received two different orphan drug designations (ODD) from the U.S. FDA. The first, for the treatment of *NR3E3* mutation-associated retinal degeneration and, most recently, for the treatment of *CEP290* mutation-associated retinal disease.

"We are delighted to partner with Ocugen as they advance their portfolio of AAV-based gene therapies for rare retinal diseases", said Dr. Xuefeng Yu, the Chairman and Chief Executive Officer of CanSinoBIO. "Our expertise in viral vector platform technologies, product development and manufacturing capabilities will play critical roles to advance OCU400 to the clinic and ultimately to serve patients in desperate need for retinal disease therapies."

About OCU400

OCU400 is a novel gene therapy with the potential to be broadly effective in restoring retinal integrity and function across a range of genetically diverse inherited degenerative retinal diseases. OCU400 is the first program that Ocugen is advancing based on its novel modifier gene therapy platform, developed by Neena Haider, PhD, Associate Professor of Ophthalmology at Harvard Medical School and Associate Scientist at the Schepens Eye Research Institute of Massachusetts Eye and Ear, from which Ocugen obtained an exclusive worldwide license to develop and commercialize ophthalmology products based on the platform. Consisting of a functional copy of the nuclear hormone receptor (NHR) gene *NR2E3*, OCU400 is delivered to target cells in the retina using an adeno-associated viral (AAV) vector. As a potent modifier gene, expression of *NR2E3* within the retina may help reset retinal homeostasis, stabilizing cells and potentially rescuing photoreceptors from degeneration.

About Ocugen, Inc.

Ocugen, Inc. is a clinical stage biopharmaceutical company focused on discovering, developing and commercializing a pipeline of innovative therapies that address rare and underserved eye diseases. The Company offers a robust and diversified ophthalmology portfolio that includes novel gene therapies, biologics, and small molecules and targets a broad range of high-need retinal and ocular surface diseases. Ocugen is leveraging its groundbreaking modifier gene therapy platform to address genetically diverse inherited retinal disorders and dry AMD, based on nuclear hormone receptor genes *NR2E3* (OCU400) and *RORA* (OCU410), respectively. OCU400 has received two orphan drug designations (ODD) targeting two distinct IRDs. Ocugen is also developing novel biologic therapies for wet-AMD, DME and diabetic retinopathy (OCU200), as well as for retinitis pigmentosa (OCU100). The Company's late-stage Phase 3 trial for patients with ocular graft versus host disease (oGVHD)(OCU300) leverages Ocugen's patented OcuNanoE – Ocugen's ONE Platform™ technology to enhance the efficacy of topical ophthalmic therapeutics. OCU300 is the first and only therapeutic with ODD for oGVHD, providing certain regulatory and economic benefits. For more information, please visit www.ocugen.com.

About CanSino Biologics Inc.

Incorporated in 2009 in Tianjin, China, CanSinoBIO (6185.HK) commits to research, development, production and commercialization of innovative vaccines for China and global public health. It possesses four integrated platform technologies including viral vectors, conjugation, protein design and recombination and formulation. As of today, it has established a robust pipeline of 15 candidate vaccines covering 12 diseases, including a globally innovative Ebola virus disease vaccine approved for emergency use and stockpile in 2017. For more information, please visit www.cansinotech.com.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from the Company's current expectations. These and other risks and uncertainties are more fully described in our periodic filings with the Securities and Exchange Commission (the "SEC"), including the risk factors described in the section entitled "Risk Factors" in Histogenics' Registration Statement on Form S-4 (Reg. No. 333-232147), as amended, filed with the SEC. Any forward-looking statements that the Company makes in this press release speak only as of the date of this press release. The Company assumes no obligation to update forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20190930005236/en/>

Source: Ocugen, Inc.

[Ocugen, Inc.](#)

Kelly Beck

kelly.beck@ocugen.com

+1 484-328-4698