



Nature Gene Therapy Publishes Preclinical Data of Ocugen's OCU400 (NR2E3-AAV) Genetic Modifier to treat Retinitis Pigmentosa (RP)

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Data support nuclear hormone receptor gene NR2E3 as genetic modifier and therapeutic agent to treat multiple retinal degenerative diseases and potentially serve as a broad-spectrum therapy

MALVERN, Pa., March 03, 2020 (GLOBE NEWSWIRE) -- [Ocugen Inc.](#) (NASDAQ: OCGN), a clinical-stage company focused on discovering, developing and commercializing transformative therapies to treat rare and underserved ophthalmic diseases, announced today the publication in *Nature Gene Therapy* of preclinical data of nuclear hormone receptor gene *NR2E3* as a genetic modifier and therapeutic agent to treat multiple retinal degenerative diseases. OCU400 (*NR2E3*-AAV) has received two orphan drug designations targeting two distinct inherited retinal diseases (IRDs): *NR2E3* mutation-associated retinal diseases and *CEP290* mutation-associated retinal diseases.

The publication details efficacy results in five unique mouse models of retinitis pigmentosa (RP) that underwent administration of *NR2E3*-AAV by subretinal injection. The five RP models tested were *rd1* (*PDE6β* associated RP), *Rho*^{-/-} and *Rho*^{P23H} (both Rhodopsin associated RP), *rd16* (Leber Congenital Amaurosis) and *rd7* (Enhanced S-cone Syndrome). The study demonstrates the potency of a novel modifier gene therapy to elicit broad-spectrum therapeutic benefits in early and intermediate stages of RP.

Please refer to [Nature Gene Therapy's online publication](#) for additional results from this study.

"This represents an important milestone for the development of this therapy. I am impressed by the protection that was elicited in multiple animal models of degeneration caused by different mutations. A treatment for inherited retinal degenerations that is mutation independent would have wide reaching implications," said Mark Pennesi, M.D., Ph.D., Associate Professor of Ophthalmology at the Oregon Health & Science University (OHSU) School of Medicine and Division Chief of the Ophthalmic Genetics Service at the OHSU Casey Eye Institute.

"Dr. Haider and her vision research team have successfully demonstrated proof of principle in their elegant study by rescuing 5 animal models of RP by resetting homeostasis. This is the foundation work for the development of the first broad spectrum therapy for inherited retinal degeneration diseases and is a game changer for rescue even after disease onset," said Cheryl Mae Craft, Ph.D., Professor of Ophthalmology, USC Keck School of Medicine at USC Roski Eye Institute Los Angeles, CA.

"One of the biggest advantages of our modifier gene therapy platform is that it has the potential to eliminate the need for individual gene replacement and gene editing strategies and may revolutionize gene therapy treatments for eye diseases. Inherited retinal degenerations such as RP affect over 1.5 million people worldwide. Over 150 gene mutations have been associated with RP and this number represents only 60% of the RP population. The remaining 40% of RP patients cannot be genetically diagnosed, making it difficult to develop individual treatments. Our modifier gene therapy has potential to eliminate the need for developing more than 150 individual products and provide one treatment option for all RP patients," said Rasappa Arumugham, Ph.D., Ocugen's Chief Scientific Officer. "We are completing preclinical studies for OCU400 and anticipate commencing a Phase 1/2a clinical trial in patients in 2021."

About Retinitis Pigmentosa

Retinitis Pigmentosa (RP) is a group of rare, genetic disorders that involve a breakdown and loss of cells in the retina. According to the National Eye Institute, it is estimated that RP affects approximately 1 in 4,000 people, both in the U.S. and worldwide. RP symptoms often begin in childhood and progress over time. Children often have difficulty getting around in the dark and as their symptoms progress, lose their peripheral (side) vision and eventually experience vision loss and blindness. Because there are many gene mutations that cause this disorder, its progression can differ greatly from person to person.

About OCU400

OCU400 is a novel gene therapy product candidate with the potential to be broadly effective in restoring retinal integrity and function across a range of genetically diverse IRDs. OCU400 is the first program that Ocugen is advancing based on its breakthrough modifier gene therapy platform developed by Dr. 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. Ocugen obtained an exclusive worldwide license from SERI to develop and commercialize ophthalmology products based on the platform. Consisting of a functional copy of the nuclear hormone receptor 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 transformative therapies to treat the whole eye. Our Phase 3 small molecule drug candidate for ocular graft-versus-host disease, if approved, will be the first and only treatment for this orphan disease. Our breakthrough modifier gene therapy platform has the potential to treat multiple retinal diseases with one drug – "one to many". And our novel biologic product candidate aims to offer better therapy to patients with underserved diseases such as wet age-related macular degeneration, diabetic macular edema and diabetic retinopathy. For more information, please visit www.ocugen.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, which are subject to risks and uncertainties. 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 the Company’s Quarterly Report on Form 10-Q filed with the SEC on November 12, 2019. Any forward-looking statements that the Company makes in this press release speak only as of the date of this press release. Except as required by law, the Company assumes no obligation to update forward-looking statements contained in this press release whether as a result of new information, future events or otherwise, after the date of this press release.

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