Ocugen Granted FDA Orphan Drug Designation for OCU400 (AAV-hNR2E3) Gene Therapy for the Treatment of RHO Mutation-Associated Retinal Degenerative Disease

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Third orphan drug designation for the same product, OCU400, is unique in Ophthalmology gene therapy and demonstrates its potential to treat many Inherited Retinal Degenerative diseases (IRDs)

MALVERN, Pa., July 27, 2020 (GLOBE NEWSWIRE) -- Ocugen, Inc. (NASDAQ: OCGN), a biopharmaceutical company focused on discovering, developing, and commercializing transformative therapies to cure blindness diseases, today announced the U.S. Food and Drug Administration (FDA) granted the third Orphan Drug Designation (ODD) for OCU400 in the treatment of *RHO* mutation-associated retinal degeneration. The *RHO* mutation is part of the Retinitis Pigmentosa (RP) group of rare, genetic disorders that involve a breakdown and loss of cells in the retina and can lead to visual impairment and blindness. This is one of the larger mutations within the RP class, representing about 12% of RP patients in the US.

A novel gene therapy product candidate, OCU400 has the potential to be broadly effective in restoring retinal integrity and function across a range of genetically diverse inherited retinal diseases. It consists of a functional copy of a nuclear hormone receptor (NHR) gene, NR2E3, delivered to target cells in the retina using an adeno-associated viral vector. As a potent modifier gene, expression of NR2E3 within the retina may help reset retinal homeostasis and potentially offer longer benefit, stabilizing cells and rescuing photoreceptor degeneration and vision loss.

Adding to ODDs for OCU400 for NR2E3 and CEP290 mutation-associated retinal degeneration, *RHO* gene mutation-associated retinal degeneration further supports Ocugen's breakthrough modifier gene therapy platform's potential to treat multiple blindness diseases with a single product. RP is a group of heterogenic inherited retinal diseases associated with over 150 gene mutations, affecting over 1.5 million individuals worldwide. In addition, ~40% of RP patients cannot be genetically diagnosed, confounding the ability to develop personalized RP therapies. Traditional gene therapy or gene editing approaches may require more than 150 products to rescue these patients from vision loss. OCU400, a single product candidate, has potential to address broad-spectrum RP.

"OCU400, comprising the nuclear hormone receptor gene *NR2E3*, has the potential to help modulate numerous biological pathways that function in maintaining the health of the retina. A recent preclinical study published in ***Nature Gene Therapy*** demonstrated the potency of *NR2E3* to elicit broad-spectrum therapeutic benefits in early and intermediate stages of RP in five unique mouse models," said Dr. Mohamed Genead, acting Chief Medical Officer of Ocugen and Chair of Ocugen's Retina Scientific Advisory Board. "We believe OCU400 has the potential to address multiple genetic mutations associated with RP and, therefore, help a broader pool of patients," Dr. Genead continued.

"Our third ODD for OCU400 from the FDA is an important step towards developing a broad-spectrum treatment for RP and getting a therapy faster to patients who are in desperate need of rescue," said Dr. Shankar Musunuri, Chairman, Chief Executive Officer and Co-Founder of Ocugen. "Orphan designation for this indication supports the goal of our Modifier Gene Therapy Platform to treat a variety of inherited retinal diseases with a single gene therapy product. There are currently no approved treatments which slow or stop the progression of multiple forms of RP, which is why we're excited to have a platform that can potentially address multiple mutations, including mutations in the Rhodopsin gene, with one therapy."

The FDA Office of Orphan Products Development grants orphan designation for novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. Orphan designation qualifies the sponsor of the drug for various development incentives of the Orphan Drug Act, including a seven-year period of U.S. marketing exclusivity, tax credits for clinical research costs, clinical research trial design assistance, the ability to apply for annual grant funding and waiver of Prescription Drug User Fee Act filing fees.

About OCU400
OCU400 (AAV-hNR2E3) 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 inherited retinal diseases. It consists of a functional copy of a nuclear hormone receptor gene, NR2E3, delivered to target cells in the retina using an adeno-associated viral vector. As a potent modifier gene, expression of NR2E3 within the retina may help reset retinal homeostasis, potentially stabilizing cells and rescuing photoreceptor degeneration and vision loss.

About Ocugen, Inc.
Ocugen, Inc. is a biopharmaceutical company focused on discovering, developing, and commercializing transformative therapies to cure blindness diseases. 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 **https://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 our 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 quarterly and annual reports that we file with the SEC. Any forward-looking statements that we make in this press release speak only as of the date of this press release. Except as required by law, we assume 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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