Ocugen Receives Fourth FDA Orphan Drug Designation for the Same Product, OCU400 (AAV-NR2E3) Gene Therapy, for the Treatment of Another Key Inherited Retinal Disease (IRD), PDE6B Gene Mutation-Associated Retinal Diseases

August 10, 2020

Fourth orphan drug designation for the same product, OCU400, is distinct in Ophthalmology gene therapy and demonstrates its potential to treat many Inherited Retinal Diseases

MALVERN, Pa., Aug. 10, 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 fourth Orphan Drug Designation (ODD) for OCU400 in the treatment of *PDE6B* gene mutation-associated retinal diseases. Retinitis Pigmentosa (RP) caused by *PDE6B* mutation is an inherited retinal dystrophy that leads to blindness by midlife and is characterized by the progressive loss of photoreceptors, with or without the loss of retinal pigment epithelium cells. At least one mutation in the *PDE6B* gene has been found to cause autosomal dominant congenital stationary night blindness, which is characterized by the inability to see in low light.

Ocugen's Modifier Gene Therapy Platform offers a unique approach in ophthalmology by addressing multiple diseases with a single product. 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. In pre-clinical studies, OCU400 has demonstrated improved vision signals in the retina where Electroretinogram response reveals rescue under both Scotopic (dim-lit) as well as Photopic (well-lit) conditions. The Company believes targeting multiple diseases with one product could also offer a smoother regulatory pathway and the ability to recover development costs over multiple therapeutic indications. Ocugen is planning to initiate two parallel Phase I/II clinical trials next year targeting two unique IRDs.

Following up on recent announcement of an ODD for *RHO* mutation-associated retinal degeneration and previous ODDs for both *NR2E3* and *CEP290* mutation-associated retinal degeneration, the ODD for *PDE6B* gene mutation-associated retinal degeneration continues to support 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.

"As principal investigator of numerous major clinical trials developing new medical and surgical treatments for retinal disorders, I have been on the cutting-edge of many new ophthalmology treatments. I am very encouraged by the potential for OCU400 given the uniqueness of Ocugen's Modifier Gene Therapy Platform and the fact that FDA has issued four ODDs for this product. I look forward to Ocugen commencing clinical trials for OCU400 next year and the potential of helping patients by restoring retinal integrity and function across a range of genetically diverse inherited retinal diseases including broad-spectrum RP," said Carl D. Regillo, M.D.,F.A.C.S., member of Ocugen's Retina Scientific Advisory Board and Professor of Ophthalmology at the Sidney Kimmel Medical College at Thomas Jefferson University, Chief of the Retina Service at Wills Eye Hospital and founder and former director of the Wills Eye Clinical Retina Research Unit in Philadelphia.

"I am thrilled to announce our fourth ODD for OCU400 from the FDA after announcing our third ODD for RHO mutation-associated retinal degeneration just a few days ago. With no approved treatments that slow or stop the progression of RP, we are dedicated to driving the development of our Modifier Gene Therapy Platform forward and potentially addressing the unmet need of multiple gene mutations, including mutations in the *PDE6B* gene with only one product," said Dr. Shankar Musunuri, Chairman, Chief Executive Officer and Co-Founder of Ocugen.

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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