European Commission Grants Ocugen Orphan Medicinal Product Designation for Gene Therapy Product Candidate, OCU400, For the Treatment of Both Retinitis Pigmentosa and Leber Congenital Amaurosis

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MALVERN, Pa., Feb. 23, 2021 (GLOBE NEWSWIRE) -- Ocugen, Inc., (NASDAQ: OCGN), a biopharmaceutical company focused on discovering, developing, and commercializing gene therapies to cure blindness diseases and developing a vaccine to save lives from COVID-19, today announced that on the recommendation of the European Medicines Agency (EMA), the European Commission has granted orphan medicinal product designation for OCU400 (AAV5-h*NR2E3*), for the treatment of both retinitis pigmentosa (RP) and Leber Congenital amaurosis (LCA).

The prevalence of RP in Europe is estimated at approximately 165,000 patients and the prevalence of LCA in Europe is estimated at approximately 40,000 patients. Globally, the number of people suffering from RP and LCA is estimated to be around 2.0 million and 0.2 million, respectively.

“We believe the granting of this designation by the European Commission validates the potential of our modifier gene therapy platform to treat many inherited retinal diseases (IRDs). IRDs associated with RP and LCA diseases are caused by mutations in over 175 genes, and it is impractical to develop therapies that are specific to each gene. OCU400 has the remarkable potential to address a significant number of patients globally who are in desperate need of rescue from these blindness diseases and we are working diligently to move this program to clinic,” said Dr. Shankar Musunuri, Chairman of the Board, Chief Executive Officer, and Co-founder of Ocugen.

“RP and LCA are chronically debilitating groups of IRDs characterized by severe impairment in visual functions starting as young as infancy, often progressing into night blindness and tunnel vision and eventually causing total blindness as early as the patient’s mid-40s. Since the existing approved therapy only addresses a small percentage of this population, there is an unmet need for new treatment options addressing a wider population of patients with IRDs,” said Dr. Mohamed Genead, Chair of Retina Scientific Advisory Board and Acting Chief Medical Officer of Ocugen.

Nuclear Hormone Receptors such as *NR2E3* are important modulators of retinal development and function acting as “master genes” in the retina. *NR2E3* 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, potentially stabilizing cells and rescuing photoreceptor degeneration. Preclinical results published in *Nature Gene Therapy* demonstrate the potency of modifier gene therapy to elicit broad-spectrum therapeutic benefits in early and advanced stages of RP including vision rescue in early and advanced stages of the disease.

Orphan medicinal product designation in Europe offers certain benefits to drug developers while they develop drugs intended for safe and effective treatment, diagnosis, or prevention of rare diseases or conditions that impact fewer than 5 in 10,000 patients in the European Union. Benefits include protocol assistance, reduced regulatory fees, research grants, and 10 years of market exclusivity following regulatory approval.

About Retinitis Pigmentosa
Retinitis pigmentosa is a clinically and genetically heterogeneous group of IRDs characterized by diffuse progressive dysfunction of predominantly rod photoreceptors, with subsequent degeneration of cone photoreceptors, and retinal pigment epithelium (RPE). Visual impairment usually manifests as night blindness and progressive visual field loss. Its prevalence is 1 in 3,000 to 1 in 5,000. RP may be seen in isolation (typical RP) or in association with systemic disease. 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.

About Leber Congenital Amaurosis
Leber congenital amaurosis is a family of congenital retinal dystrophies that results in severe vision loss at an early age. Patients present usually with nystagmus, sluggish or near-absent pupillary responses, severely decreased visual acuity, photophobia and high hyperopia. It is the most severe retinal dystrophy causing blindness by the age of 1 year. This dystrophy is a genetically heterogeneous recessive disease affecting 1 in 30,000 to 1 in 81,000 subjects. Mutations in one of more than two dozen genes can cause LCA.

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, Associate Professor of Ophthalmology at Harvard Medical School and Associate Scientist at the Schepens Eye Research Institute (SERI) of Massachusetts Eye and Ear. Ocugen obtained an exclusive worldwide license from SERI to develop and commercialize ophthalmology products based on specified nuclear hormone receptor genes, including *NR2E3*. Consisting of a functional copy of the nuclear hormone receptor gene *NR2E3*, OCU400 is delivered to target cells in the retina using an 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 biopharmaceutical company focused on discovering, developing, and commercializing gene therapies to cure blindness diseases and developing a vaccine to save lives from COVID-19. 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. We are co-developing Bharat Biotech’s COVAXIN™ vaccine candidate for COVID-19 in the U.S. market. 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 our current expectations. These and other risks and uncertainties are more fully described in our periodic filings with the Securities and Exchange Commission (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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