Ocugen Granted FDA Orphan Drug Designation for OCU400 Gene Therapy for the Treatment of NR2E3 Mutation-Associated Retinal Degenerative Disease

February 14, 2019

MALVERN, Pa., Feb. 14, 2019 /PRNewswire/ -- Ocugen, Inc., a clinical stage biopharmaceutical company focused on discovering, developing and commercializing a pipeline of innovative therapies that address rare and underserved eye diseases, today announced the U.S. Food and Drug Administration (FDA) granted orphan drug designation (ODD) for OCU400, Ocugen's novel gene therapy, for the treatment of *NR2E3* mutation-associated retinal degenerative disease. Inherited retinal diseases (IRDs) are caused by genetic mutations that are passed down within families and lead to visual impairment and blindness. OCU400 consists of adeno-associated virus serotype 5 capsid containing the gene for human Nuclear Hormone Receptor *NR2E3* and is the first program that Ocugen is advancing based on its modifier gene therapy platform.

"We are very pleased to receive our first orphan drug designation for a gene therapy product from the FDA," said Shankar Musunuri, PhD, MBA, Chairman, Chief Executive Officer and Co-Founder of Ocugen. "Unlike single-gene replacement approaches, which have shown great promise in rare retinal diseases despite being highly specific for a single condition, we believe OCU400 represents a powerful and broad means of treating a variety of genetically diverse IRDs with a single product. Our initial strategy is to develop OCU400 as a gene augmentation therapy for patients with IRDs caused by mutations in the *NR2E3* gene. Orphan designation for this initial indication is not only a major milestone for our Modifier Gene Therapy Platform, it also marks the third product in Ocugen's clinical pipeline to be granted ODD. Our other orphan drug candidates include OCU100 for retinitis pigmentosa and OCU300, which is currently in Phase 3 clinical trials for the treatment of ocular graft versus host disease and is the only product candidate to be granted ODD for this indication."

In a mouse model of NR2E3 mutation, NR2E3 delivery to retinal cells reversed disease progression. Based on these encouraging results, Ocugen plans to initiate a Phase 1/2a clinical study by 2020. Future opportunities for OCU400 include additional rare/orphan retinal diseases where local *NR2E3* gene augmentation may effectively prevent disease progression, including Leber Congenital Amaurosis (LCA), retinitis pigmentosa and other retinal degenerative diseases.

"It is gratifying that a clinical trial is being planned for this serious retinal disorder caused by *NR2E3* mutations; it has been 30 years since we first identified this IRD and now is the time to move toward therapy," commented Samuel G. Jacobson, MD, PhD, Professor of Ophthalmology of the University of Pennsylvania.

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 (PDUFA) filing fees.

About OCU400
OCU400 is a novel gene therapy with the potential to be broadly effective restoring retinal integrity and function across a range of genetically diverse inherited degenerative retinal disease (IRDs). 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 world-wide 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 photoreceptor degeneration. Ocugen plans to initiate a Phase 1/2a clinical study for OCU400 by 2020.

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's lead clinical candidate (OCU300) is currently in Phase 3 for patients with ocular graft versus host disease (oGVHD) and is the first and only therapeutic with orphan drug designation for oGVHD, providing certain regulatory and economic benefits. The Company's second lead candidate (OCU310) is also in Phase 3 for patients with dry eye disease. Both OCU300 and OCU310 leverage Ocugen's patented OcuNanoE – Ocugen's ONE Platform™ technology to enhance the efficacy of topical ophthalmic therapeutics. Ocugen is also developing a groundbreaking modifier gene therapy platform with the potential to address a broad spectrum of inherited retinal disorders, including its lead program, OCU400 for treating *NR2E3* mutation-associated retinal degeneration, for which the Company has orphan drug designation. Additionally, Ocugen is advancing biologic therapies for Wet AMD (OCU200) and retinitis pigmentosa (OCU100). For more information, please visit www.ocugen.com.

Contact:
Ocugen, Inc.
Kelly Beck
kelly.beck@ocugen.com
+1 484-328-4698

SOURCE Ocugen, Inc.