Ocugen Receives FDA Orphan Drug Designation for OCU300 (brimonidine tartrate) for the Treatment of Ocular Graft Versus Host Disease

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FDA Decision Marks First Orphan Drug Designation for Treatment of oGVHD in the U.S.

MALVERN, Pa., Aug. 9, 2017 /PRNewswire/ -- Ocugen, Inc., a clinical stage biopharmaceutical company developing novel treatments for sight-threatening diseases, today announced the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) for OCU300 (brimonidine tartrate) for the treatment of ocular graft versus host disease (oGVHD). Ocular GVHD is a common complication that occurs in 40-60 percent of patients who have undergone allergenic hematological stem cell transplantation (allo-SCT) or bone marrow transplants. Driven by autoimmune inflammation, oGVHD induces severe ocular surface disease, which over time significantly diminishes quality of life, and restricts daily activities due to visual impairment.

"We are very excited to receive the first ever orphan drug designation by the FDA for oGVHD, emphasizing the unmet medical need for patients with this disease. This is a significant milestone that will allow us to further advance the clinical development of OCU300, with a proprietary nanoemulsion, into a phase 3 clinical trial in the near future," said Shankar Musunuri, PhD, MBA, chairman, CEO and co-founder of Ocugen.

Ocugen executed an exclusive worldwide license agreement for OCU300 with the University of Illinois at Chicago last year. Dr. Sandeep Jain, Director of the ocular GVHD Clinic at the University of Illinois at Chicago and an inventor of OCU300 for the treatment of oGVHD, said, "I'm pleased that Ocugen has received orphan drug designation for oGVHD. This is a debilitating condition for which no approved pharmaceutical therapeutics exist."

The FDA Office of Orphan Products Development (OOPD) grants orphan designation for novel drugs or biological 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 (ODA), including 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) for filing fees.

About OCU300
OCU300 is a re-purposed drug being developed through the FDA's 505(b)(2) pathway for the treatment of oGVHD. According to a post-hoc analysis of OCU300 administered to patients with oGVHD in an exploratory observational study, there was beneficial effect in approximately 90 percent of patients without significant side effects.

About Ocugen, Inc.
Ocugen is a biopharmaceutical company focused on advancing two novel biologicals and a marketed drug product as a re-purposed drug under the U.S. Food and Drug Administration's 505(b)(2) regulatory pathway to treat sight threatening ocular disorders. Our programs are focused on activating novel biologic pathways to treat inflammatory, degenerative, and neovascular diseases of the eye. OCU100 is a recombinant N-terminal fragment of Lens Epithelium Derived Growth Factor (LEDGF). Its second asset, OCU200, is an anti-angiogenic tumstatin fusion protein being developed for treatment of wet age-related macular degeneration (AMD). OCU300 is being developed through the FDA's 505(b)(2) pathway for the treatment of ocular graft versus host disease (oGVHD). All three products have a strong international patent portfolio.

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