Ocugen Initiates Phase 3 Clinical Trial of OCU300 for the Treatment of Ocular Graft Versus Host Disease

July 2, 2018

MALVERN, Pa., July 2, 2018 /PRNewswire/ -- Ocugen, Inc., a rapidly growing ophthalmology company developing a rich clinical pipeline of innovative therapies that address rare and underserved ocular diseases, today announced that it has initiated the first of two pivotal, Phase 3 clinical trials of OCU300 for the treatment of ocular discomfort and ocular redness in patients with ocular graft versus host disease (oGVHD) following recent acceptance of Ocugen's New Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA). OCU300 consists of FDA-approved brimonidine tartrate formulated in a proprietary nanoemulsion based on Ocugen's patented OcuNanoE™ technologyOcugen is the first and only company to advance a therapy into a Phase 3 clinical trial for treating patients with oGVHD and OCU300 is the only product candidate to be granted Orphan Drug Designation for oGVHD by the FDA.

oGVHD is a severe ophthalmic comorbidity that affects about 60% of patients who undergo allogeneic bone marrow transplant, yet there is currently no approved treatment. It is a chronic and debilitating autoimmune condition characterized by painful, dry, tearless eyes, vision issues and potential ocular scarring. The proof of concept for using brimonidine to treat oGVHD was established by two previous investigator-led clinical studies. In an exploratory observational study, approximately 90% of oGVHD patients treated for six months with brimonidine eye drops reported an improvement in their symptoms without any significant side effects. In a randomized, placebo-controlled, double-masked Phase 1/2 study, oGVHD patients using brimonidine eye drops had a significant reduction in ocular redness scores ($p<0.05$) compared to patients using placebo, as well as a clinically meaningful reduction in ocular discomfort after only three months of treatment.

Daniel Jorgensen, MD, MPH, Chief Medical Officer of Ocugen, said, "We are excited to advance OCU300 into pivotal-stage clinical development and to potentially deliver to oGVHD patients the first FDA-approved therapy to address this condition. The preclinical efficacy data we presented last month at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting further highlight the ability of OCU300, in particular its novel nanoemulsion formulation, to inhibit the underlying pathophysiological processes associated with oGVHD. We believe OCU300 has significant potential to improve the quality of life of patients living with this debilitating disease."

Shankar Musunuri, PhD, MBA, Chairman, CEO and Co-Founder of Ocugen, commented, "This is a significant milestone for Ocugen and I would like to thank our dedicated team and our participating clinical sites for their work, which has enabled us to reach this stage and commence Phase 3 development in a highly efficient manner. As we begin enrolling oGVHD patients in this study, we look forward to continuing that efficiency and reporting top-line data as soon as it is available, which we currently expect to be in the second half of 2019. We also expect to launch pivotal studies for our second lead candidate, OCU310 for the treatment of dry eye disease, later this year."

About the OCU300 Phase 3 Clinical Program
The pivotal clinical program consists of two randomized, double-masked, placebo-controlled Phase 3 studies designed to examine the efficacy of OCU300 in treating ocular discomfort and redness as a result of oGVHD. The studies will be staggered, and each will evaluate approximately 60 patients randomized 2:1 to receive OCU300 or placebo (ophthalmic saline). Patients will receive daily eye drops of their assigned treatment for a period of 84 days. The primary efficacy endpoints will measure ocular discomfort using the visual analog scale (VAS) and ocular redness using the Validated Bulbar Redness (VBR) grading scale.

About OCU300
OCU300 is in pivotal stage clinical development for treating ocular discomfort and ocular redness in patients with the debilitating autoimmune condition called ocular graft versus host disease (oGVHD), which develops in many patients following an allogeneic bone marrow transplant. It is the only product to be granted Orphan Drug Designation for this indication from the U.S. FDA, and it consists of an improved 0.18% ophthalmic nanoemulsion of brimonidine tartrate, an FDA-approved drug with established safety for ocular use, enabling Ocugen to develop OCU300 under the accelerated 505(b)(2) regulatory pathway. Ocugen's patented OcuNanoE™ technology is designed to enhance efficacy by prolonging retention of this potent anti-inflammatory drug on the eye surface. In addition, it allows OCU300 to be sterile filtered into single-use vials as preservative-free nanoemulsion, thereby eliminating potentially irritating effects of preservatives.

About Ocugen, Inc.
Ocugen, Inc., is a rapidly growing ophthalmology company developing a rich clinical pipeline of innovative therapies that address rare and underserved ocular disorders. The Company's lead clinical candidate (OCU300) is currently in Phase 3 for treating 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 for treating dry eye disease (OCU310) is also expected to enter pivotal clinical trials in 2018. 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 novel biologic therapies for retinitis pigmentosa (OCU100) and wet AMD (OCU200), as well as a groundbreaking modifier gene therapy platform with potential to address a broad spectrum of inherited retinal disorders (OCU400). For more information, please visit www.ocugen.com.

Contact:
Ocugen, Inc.
Kelly Beck

kelly.beck@ocugen.com
+1 484-328-4698

SOURCE Ocugen, Inc.