Ocugen Reports Positive Phase 2 Clinical Results Demonstrating Proof-of-Concept for its Novel Combination Therapy for Dry Eye Disease

March 20, 2018

MALVERN, Pa., March 20, 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 positive results from its Phase 2 proof-of-concept clinical trial of OCU310, a novel combination of brimondine tartrate and a corticosteroid, loteprednol etabonate, being developed as a treatment for dry eye disease. The randomized, multi-center, double-blinded, placebo-controlled study met its primary endpoint of tolerability over a 12-week period. The study results also showed meaningful improvements across a number of endpoints related to the signs and symptoms of dry eye disease when compared to placebo. Based on these encouraging results, Ocugen remains on track to advance OCU310 into Phase 3 clinical studies in the third quarter 2018.

Daniel Jorgensen, M.D., MPH, Chief Medical Officer of Ocugen, stated, "We are pleased to have met our primary objective of showing tolerability and consistently greater reductions in key exploratory efficacy endpoints, especially potential sign and symptom endpoints for Phase 3 studies. We believe OCU310 can provide significant benefit to those suffering from dry eye disease, and we look forward to presenting the full results at a future academic meeting, and discussing with the FDA in the coming months."

Shankar Musunuri, Ph.D., MBA, Chairman, CEO and Co-Founder of Ocugen, commented, "We are very encouraged by these results. We believe key potential differentiators for OCU310, such as rapid onset of action and the unique potential for OCU310 to relieve dry eye discomfort, as well as potentially enhanced tolerability to support its long-term use, will drive favor for OCU310 among prescribers and patients. We anticipate further differentiating OCU310 as we move into Phase 3 studies, utilizing our enhanced and proprietary nanoemulsion preservative-free formulation of brimonidine and loteprednol in single use vials."

About OCU310
OCU310 is a unique ophthalmic nanoemulsion/suspension product being developed as a treatment for dry eye disease. It contains a proprietary nanoemulsion of brimonidine tartrate (0.2%), an FDA-approved ophthalmic drug with unique anti-inflammatory, immunosuppressive, vasoconstrictive and analgesic properties, combined with a low dose of loteprednol etabonate (0.2%), an FDA approved corticosteroid for ophthalmic use. The fact that both active components of the OCU310 combination product are approved by FDA for ophthalmic use, significantly derisks OCU310 from a product safety and regulatory (505(b)(2) pathway) standpoint. These active components, which have complementary mechanisms of action, could result in an enhanced product profile compared to currently marketed products for dry eye. OCU310 is expected to have a more rapid onset of action, improved tolerability and more potent relief from dry eye signs and symptoms due to its novel nanoemulsion formulation of brimondine combined with loteprednol.

About the OCU310 Phase 2 Proof-of-Concept Trial Results
In this Phase 2, randomized, multicenter, placebo-controlled, double-blinded study, patients with dry eye disease received 0.2% brimonidine tartrate alone or with 0.2% loteprednol etabonate (OCU310) or placebo. Patients were treated with eye drops twice daily (BID) for a period of 12 weeks. All patients were assessed for tolerability using a visual analog scale (VAS), and the results showed that tolerability was similar for patients receiving OCU310 or placebo at all post-baseline visits through week 12, fulfilling the study's primary endpoint. In addition, overall adverse event rates were low and similar to placebo, supporting the safety and tolerability of OCU310. The study was not powered to show statistical significance for efficacy, but prespecified exploratory efficacy endpoints to assess changes in key signs and symptoms of dry eye disease were evaluated. Of the symptom endpoints, the Symptom Assessment Questionnaire in Dry Eye (SANDE), which measured the frequency and severity of eye dryness/irritation, was the most relevant. SANDE scores improved from baseline at all subsequent time points, with consistently greater reductions in SANDE score for the OCU310 group compared to patients who received placebo, indicating a greater degree of relief from dry eye discomfort. As for sign endpoints, conjunctival staining with lissamine green (only measured at 12-week time point) showed a greater reduction from baseline staining for patients receiving OCU310 compared to the placebo group. Results were similar for corneal lissamine green staining, indicating greater improvement over placebo in a key sign of dry eye disease.

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 programs in ocular graft versus host disease (OCU300) and dry eye disease (OCU310) are expected to enter pivotal clinical trials in 2018. OCU300 received the first and only orphan drug designation for ocular graft versus host disease, providing certain regulatory and economic benefits. 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 Morello
kelly.morello@ocugen.com
+1 484-328-4698

SOURCE Ocugen, Inc.