Ocugen Presents Preclinical Efficacy Data of its Proprietary Nanoemulsion Technology at ARVO 2018

May 2, 2018

MALVERN, Pa., May 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 presented additional preclinical findings in a poster highlighting the potential efficacy of its unique, patented nanoemulsion formulation of brimonidine tartrate (OCU300) at the Association for Research in Vision and Ophthalmology (ARVO) 2018 Annual Meeting, held April 29 – May 3, 2018, in Honolulu, Hawaii. The results highlight superior (statistically significant) efficacy of OCU300 in a surrogate mouse model of ocular graft versus host disease (oGVHD), as compared to placebo, untreated control, and marketed brimonidine. OCU300 is in Phase 3 clinical development for treating oGVHD and is the only product to be granted Orphan Drug Designation for this indication from the U.S. FDA.

Rasappa Arumugham, Ph.D., Chief Scientific Officer of Ocugen, stated, "The data we presented yesterday clearly demonstrate the efficacy of OCU300 in inhibiting the underlying pathophysiological processes associated with oGVHD. The data also showed that OCU300 provides efficacy that was statistically significant compared to untreated and placebo controls, as well as to the current commercial formulation of brimonidine tartrate, which is approved for treating glaucoma as a standard ophthalmic solution. These encouraging results add to a growing body of evidence that our proprietary nanoemulsion formulation technology may confer enhanced drug properties and lead to improved outcomes for ophthalmic diseases. Therefore, we believe OCU300 is a strong candidate for the treatment of oGVHD that could become the first approved therapy for this significantly underserved ocular disease, which affects 40%-60% of allogeneic stem cell transplant patients. We look forward to initiating our Phase 3 trial of OCU300 for the treatment of oGVHD later this quarter."

Daniel Jorgensen, M.D., MPH, Chief Medical Officer of Ocugen, said, "Of all the drugs tested in this study, OCU300 was the only treatment that showed a favorable response in reducing corneal surface inflammation, improvement of lacrimal gland pathology and increased mucin producing goblet cells. We hope to demonstrate similar improvements in our upcoming clinical trials on patients with oGVHD."

About OCU300
OCU300 is in late-stage clinical development as a novel treatment for the debilitating immune condition called ocular graft versus host disease, which develops in many patients following an allogeneic bone marrow transplant. It consists of an improved 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. The proprietary, patented nanoemulsion 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. Ocugen plans to confirm the efficacy of OCU300 in a controlled Phase 3 study using objective end points expected to initiate in the second quarter 2018.

About the OCU300 Preclinical Study and Findings
This study evaluated the efficacy of OCU300 in a well-recognized animal model for dry eye disease (DED), which shares important pathophysiological attributes with oGVHD, enabling it to act as a surrogate animal model for this disease. The study compared OCU300 nanoemulsion to the commercially branded dry eye products cyclosporine and lifitegrast, as well as to a marketed brimonidine tartrate product that is FDA-approved for the treatment of glaucoma. Testing OCU300 in this well-recognized animal model enables reliable comparison when measuring the inhibition of certain oGVHD pathophysiological processes that are in common with those of DED.

In the study, DED was induced by exposing the mice to a standardized desiccating environment, combined with transdermal administration of scopolamine. The following drug agents were compared: 0.05% cyclosporine ophthalmic emulsion (Restasis®, Allergan), 5% lifitegrast ophthalmic solution (Xiidra®, Shire), OCU300 nanoemulsion (0.18% brimonidine tartrate, Ocugen), placebo emulsion (Ocugen), and 0.2% brimonidine tartrate ophthalmic solution (Bausch & Lomb). All drugs were administered topically directly onto the eye surface twice daily for a period of two weeks.

By the end of the treatment period, OCU300 had improved all three functional outcomes associated with DED in this model, by decreasing corneal surface inflammation and lacrimal gland pathology, and increasing mucin-secreting conjunctival goblet cell population. These findings also suggest that OCU300 performs better compared to branded DED products (cyclosporine and lifitegrast) and marketed brimonidine tartrate.

As noted, the OCU300 nanoemulsion also demonstrated improvements in oGVHD-associated functional outcomes as compared to the marketed 0.2% brimonidine tartrate ophthalmic solution. At a 10% reduced dose, the OCU300 nanoemulsion (0.18% brimonidine tartrate) achieved a reduction in ocular surface inflammation that was statisticallysignificant ($p<0.01$) compared to the marketed 0.2% brimonidine tartrate ophthalmic solution. OCU300 also showed a positive trend with respect to reducing goblet cell loss while the 0.2% brimonidine tartrate ophthalmic solution did not differ from control groups. These improved functional outcomes in the surrogate oGVHD mouse model compared to the branded brimonidine solution appear to be due to Ocugen's enhanced proprietary nanoemulsion formulation, providing longer residence time and optimal ocular bioavailability (unpublished data).

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

SOURCE Ocugen, Inc.