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Abstract on ZETA-1 Study of APX3330 Receives Award at Women in Ophthalmology (WIO) Summer Symposium

ZETA-1 abstract rated as one of top three scoring abstracts out of nearly 600 submissions at WIO and received an award distinction

FARMINGTON HILLS, Mich., Aug. 28, 2023 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing small-molecule therapies for the treatment of retinal and refractive eye disorders, today announced that data from its Phase 2 clinical trial of APX3330 in Diabetic Retinopathy (DR) were featured in an oral presentation on Saturday, August 26th at the Women in Ophthalmology Summer Symposium, in Marco Island, Florida.

The presentation "*ZETA-1 Phase 2 Trial Safety and Tolerability Results for APX3330: A Novel, Oral Ref-1 Inhibitor for the Treatment of Diabetic Retinopathy*" was delivered by Priya Vakharia M.D., vitreo-retina specialist at Retina Vitreous Associates of Florida. The abstract received the Joanne Angle Abstract of Distinction Award out of nearly 600 submissions.

"I am honored to receive the prestigious Joanne Angle Abstract of Distinction award from WIO for this abstract on ZETA-1 data, and to have the opportunity to share these important data with my fellow ophthalmologists," said Priya Vakharia M.D. "In the ZETA-1 trial, APX3330 achieved statistical significance in preventing clinically meaningful progression of diabetic retinopathy, as measured by the percentage of subjects with binocular 3-step worsening in Diabetic Retinopathy Severity Scale (DRSS). DR is a common cause of blindness; however, the majority of NPDR patients are not actively treated today because of the burden of invasive anti-VEGF treatments. If approved, APX3330 can allow us to intervene early in the disease and prevent vision-threatening complications."

Rick Rodgers, Interim CEO of Ocuphire added. "I would like to thank WIO and Dr. Vakharia for the opportunity to present our ZETA-1 data at this year's Summer Symposium APX3330 is a non-invasive convenient oral therapy that has demonstrated favorable safety and tolerability with compelling potential to slow progression of DR. We believe that, if approved, APX3330 could fundamentally shift the treatment paradigm in DR and allow for a broad prescriber base to manage the disease. We look forward to our forthcoming End-of-Phase 2 meeting with the FDA later this year and intend to confirm our Phase 3 registration endpoint and path to a potential approval."

About Ocuphire Pharma

Ocuphire Pharma, Inc. is a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing small-molecule therapies for the treatment of retinal and refractive eye disorders.

Ocuphire's lead retinal product candidate, APX3330, is a first-in-class small-molecule inhibitor of Ref-1 (reduction oxidation effector factor-1 protein). Ref-1 is a regulator of transcription factors such as HIF-1a and NF-kB. Inhibiting REF-1 reduces levels of vascular endothelial growth factor ("VEGF") and inflammatory cytokines which are known to play key roles in ocular angiogenesis and inflammation. Through inhibition of Ref-1, APX3330 normalizes the levels of VEGF to physiologic levels, unlike biologics that abolish the VEGF levels required for normal function. APX3330 is an oral tablet administered twice per day for the treatment of diabetic retinopathy ("DR") and diabetic macular edema ("DME"). A Phase 2 study in subjects with DR or DME has recently completed, and an End-of-Phase 2 meeting is confirmed with the FDA in Q4 2023.

DR affects approximately 10 million people with diabetes and is projected to impact 14.6 million Americans by 2050. DR is classified as Non-Proliferative Diabetic Retinopathy ("NPDR"), the early stage of the disease in which symptoms may be mild or nonexistent or Proliferative Diabetic Retinopathy ("PDR") which is the more advanced stage of diabetic eye disease that can be highly symptomatic with loss of vision. Approximately 80% of DR patients have NPDR that will progress to PDR if left untreated. Despite the risk for visual loss associated with this disease, over 90% of NPDR patients currently receive no course of treatment apart from observation by their eye care specialist until they develop sight-threatening complications. This is due to the burdensome and frequent eye injections currently required with currently approved therapies for this disease. APX3330 as an oral tablet has the potential to be an early, non-invasive treatment for the 8 million NPDR patients in the US. Treatment with APX3330 is expected to delay or prevent progression of NPDR, thereby reducing the need for expensive intravitreal injections with anti-VEGF therapies and reducing the likelihood of vision loss due to DR.

Ocuphire has also in-licensed APX2009 and APX2014, which are second-generation analogs of APX3330. The unique dual mechanism of action of these Ref-1 inhibitors of reducing angiogenesis and inflammation could potentially be beneficial in treating other retinal diseases such as age-related macular degeneration ("AMD"), and geographic atrophy ("GA"). Ocuphire is currently evaluating local delivery routes in addition to the systemic (oral) route as part of its pipeline expansion in retinal therapies.

Ocuphire has a partnership with Viatris, Inc. to develop and commercialize Nyxol® eye drops as a preservative-free eye drop formulation of phentolamine mesylate, a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size by uniquely blocking the alpha-1 receptors found only on the iris dilator muscle without affecting the ciliary muscle. Nyxol has been studied in a total of 12 clinical trials across three indications, including single-use for reversal of pharmacologically-induced mydriasis ("RM"), and once-daily for treatment of presbyopia and dim light (night) vision disturbances ("DLD"), pending regulatory approvals. Nyxol's NDA under the 505(b)(2) pathway for the first indication, RM, has been accepted with a PDUFA date assigned of September 28, 2023. Nyxol is currently in Phase 3 for presbyopia and DLD.

For more information, visit www.ocuphire.com.

Forward Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements concerning the End-of-Phase 2 meeting with the FDA to confirm Phase 3 registration endpoints and study parameters, and the potential receipt of regulatory approval for Nyxol for the treatment of RM. These forward-looking statements are based upon Ocuphire's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, including, without limitation: (i) the success and timing of regulatory submissions and pre-clinical and clinical trials, including enrollment and data readouts; (ii) regulatory requirements or developments; (iii) changes to clinical trial designs and regulatory pathways; (iv) changes in capital resource requirements; (v) risks related to the inability of Ocuphire to obtain sufficient additional capital to continue to advance its product candidates and its preclinical programs; (vi) legislative, regulatory, political and economic developments, (vii) changes in market opportunities, (viii) the effects of COVID-19 on clinical programs and business operations, (ix) risks that the Nyxol partnership may not facilitate the commercialization or market acceptance of Ocuphire's product candidates; (x) the success and timing of commercialization of any of Ocuphire's product candidates, and (xi) the maintenance of Ocuphire's intellectual property rights. The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive and should be read in conjunction with statements that are included herein and elsewhere, including the risk factors detailed in documents that have been and may be filed by Ocuphire from time to time with the SEC. All forward-looking statements contained in this press release speak only as of the date on which they were made. Ocuphire undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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