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Ocuphire Pharma Announces Last Patient Completes Final Visit in ZETA-1 Phase 2b 24-Week Trial of Oral APX3330 in Diabetic Retinopathy

Top-line Results are Expected in 4Q 2022

Interim Masked Safety Results Seen Demonstrate Favorable Safety and Tolerability Profile, Consistent with 11 Prior Trials of APX3330

Oral APX3330 has the Potential to Serve a Market of More Than 8 Million Diabetic Retinopathy Patients with Limited Treatment Options

FARMINGTON HILLS, Mich., Sept. 08, 2022 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of refractive and retinal eye disorders, today announces that the last patient of the 103 enrolled subjects since April 2021 has completed their 24-week study visit in the Phase 2b ZETA-1 trial of oral APX3330 for the treatment of diabetic retinopathy (DR). Top-line results are expected in the fourth quarter of 2022.

“APX3330 is a potential first-in-class oral treatment for diabetic retinopathy/diabetic macular edema, where the need for early intervention remains high. With the final patient visit completed in this longer term study, we remain on track to report top-line results in the fourth quarter of this year, potentially bringing us one step closer to delivering the first small molecule, dual action, oral treatment option to patients with progressive vision-threatening diabetic eye disease,” said Mina Sooch, Ocuphire Pharma’s Founder and CEO. “We look forward to sharing our top-line results later this year. In advance of the top-line results, Ocuphire plans to host a retina KOL webinar to showcase APX3330 and discuss the opportunity in DR. The event is scheduled for Friday October 14, 2022, with more details coming soon.”

David Boyer, MD, Senior Partner, Retina-Vitreous Associates Medical Group said, “As an investigator in the ZETA-1 trial and a retina specialist that provides care for a large diabetic patient population, I am excited by the prospect of providing my DR/DME patients with a potential first-in-class, oral therapy with a novel dual mechanism of action of anti-inflammatory and anti-VEGF. Interim masked safety results seen to-date demonstrate favorable safety and tolerability profile, consistent with 11 prior trials of APX3330. If approved, APX3330 could represent a paradigm shift from observation and monitoring progression today to an early, non-injection treatment option.”

The multi-center, randomized, double-masked, placebo-controlled Phase 2b ZETA-1 trial

was designed to evaluate the efficacy and safety of APX3330 in diabetic retinopathy patients. The study was conducted at 25 U.S. sites and enrolled 103 subjects with moderately severe to severe non-proliferative DR (NPDR) or mild proliferative diabetic retinopathy (mild PDR). Patients were randomized to receive 600 mg APX3330 or placebo daily over 24 weeks. The trial initiated in April 2021 and completed last patient last visit in August 2022.

About Diabetic Retinopathy

Diabetes, a worldwide epidemic, is the leading cause of blindness among adults age 20 to 74. DR is the most common diabetic complication that affects the eyes and is manifested when chronically elevated blood sugar levels cause damage to blood vessels in the retina. DR affects over 8 million patients in the U.S. and 93 million patients worldwide. This problem is projected to worsen as the number of individuals at risk of developing diabetes increases by 55% by 2035.

There are two major types of DR: (1) non-proliferative DR (NPDR) and (2) proliferative DR (PDR). NPDR is an earlier, more typical stage of DR that can progress to more severe forms of DR if untreated and if the underlying diabetes remains uncontrolled. PDR is a more advanced stage of DR that is characterized by retinal neovascularization that, if left untreated, can lead to permanent damage and blindness. When DR is in its early stages, blood vessels in the retina are damaged and can leak fluid into the retina, a complication called diabetic macular edema (DME). Fluid from DME and hemorrhage of the abnormal blood vessels formed in PDR, can interfere with vision and can cause irreversible visual impairment due to retinal scarring and retinal detachment. Despite the approval of intravitreal injection therapies for DR, patients with DR are not widely treated.

About APX3330

APX3330 is a first-in-class, small molecule, oral inhibitor of the transcription factor regulator Ref-1 (reduction-oxidation effector factor-1). With a novel dual mechanism of action, APX3330 blocks the downstream pathways regulated by Ref-1 -- including those involving angiogenesis (VEGF) and inflammation (NFkB) -- to decrease abnormal activation of both angiogenesis, and of inflammatory pathways that are implicated across several ocular diseases, including DR, DME, and age-related macular degeneration (AMD).

APX3330 has shown a favorable safety and tolerability profile over 11 clinical trials conducted in healthy, hepatitis, and cancer subjects prior to the current Phase 2 ZETA-1 trial in diabetic retinopathy. The most recent interim analysis of masked safety data from ZETA-1 trial was presented by Dr. Michael Allingham, M.D., Ph.D., at the American Society of Retina Specialists' (ASRS) 40th Annual Scientific Meeting in July 2022. These data indicated that oral APX3330 continued to demonstrate a favorable safety profile consistent with the prior trials comprising hundreds of patients. Across all the trials, the safety findings represent over 9000 subject-days of exposure at the target dose of 600 mg/day.

About Ocuphire Pharma

Ocuphire is a publicly traded (Nasdaq: OCUP), clinical-stage, ophthalmic biopharmaceutical company focused on developing and commercializing small-molecule therapies for the treatment of refractive and retinal eye disorders.

The Company's lead product candidate, Nyxol[®] eye drops (0.75% phentolamine ophthalmic solution), is a once-daily, preservative-free eye drop formulation of phentolamine mesylate, a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size, and is being developed for several indications, including reversal of pharmacologically-induced mydriasis (RM), presbyopia and dim light or night vision disturbances (NVD). Nyxol has been studied in 12 completed clinical trials, with positive data reported from the MIRA-2 and MIRA-3 registration trials and the MIRA-4 pediatric safety trial for the treatment of RM. Ocuphire also reported positive top-line data from the VEGA-1 Phase 2 trial of Nyxol for treatment of presbyopia, which evaluated both Nyxol as a single agent and Nyxol with low dose pilocarpine ("LDP") 0.4% as adjunctive therapy. The Company recently announced positive top-line results from the LYNX-1 Phase 3 trial of Nyxol for night vision disturbances (NVD).

Ocuphire's second product candidate, APX3330, is an oral tablet designed to inhibit angiogenesis and inflammation pathways relevant to retinal and choroidal vascular diseases, such as diabetic retinopathy (DR) and diabetic macular edema (DME). APX3330t has been studied in 11 Phase 1 and 2 trials.

Please visit www.clinicaltrials.gov to learn more about Ocuphire's ongoing APX3330 Phase 2b trial in DR/DME ZETA-1 ([NCT04692688](https://clinicaltrials.gov/ct2/show/study/NCT04692688)) and completed Nyxol trials: Phase 3 registration trial in NVD LYNX-1 ([NCT04638660](https://clinicaltrials.gov/ct2/show/study/NCT04638660)), Phase 3 registration trials in RM MIRA-2 ([NCT04620213](https://clinicaltrials.gov/ct2/show/study/NCT04620213)) and MIRA-3 ([NCT05134974](https://clinicaltrials.gov/ct2/show/study/NCT05134974)), MIRA-4 Phase 3 pediatric safety study ([NCT05223478](https://clinicaltrials.gov/ct2/show/study/NCT05223478)), and Phase 2 trial in presbyopia VEGA-1 ([NCT04675151](https://clinicaltrials.gov/ct2/show/study/NCT04675151)). 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, the success and timing of planned regulatory filings, including the results of the ZETA-1 Phase 2b trial, the market for Ocuphire's indications including DR, business strategy, pre-commercialization activities, and commercialization of Ocuphire's product candidates. 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) the success and timing of commercialization of any of Ocuphire's product candidates and (x) 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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