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Ocuphire Completes Enrollment in VEGA-1 Phase 2 Clinical Trial Investigating Nyxol® in Combination with Low-Dose Pilocarpine for Treatment of Presbyopia

Nyxol has Potential to be a New Eye Drop Treatment Option for Presbyopia

Top-Line VEGA-1 Data Expected by End of Q2 2021

FARMINGTON HILLS, Mich., May 12, 2021 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of several eye disorders, announced today that it has completed enrollment in the VEGA-1 Phase 2 clinical trial (NCT04675151) evaluating the safety and efficacy of a combination kit of Nyxol® and low-dose pilocarpine in presbyopia. VEGA-1 successfully recruited 150 subjects in just under 3 months.

“Achieving our enrollment completion target for the VEGA-1 trial is yet another important milestone in our Nyxol program and tracks for top-line results by the end of June,” said Mina Sooch, MBA, President and CEO of Ocuphire Pharma. “Nyxol has the potential to address multiple unmet needs initially with the large market opportunity in reversal of mydriasis supported by our recent positive Phase 3 data results and then adding this very large presbyopia market opportunity that is estimated at over $5 billion in the US alone. We are excited to develop Nyxol with low-dose pilocarpine as a potential option for the growing number of presbyopic patients and to also realize commercial synergies if products are approved given the common targeted optometrists and ophthalmologists.”

The global prevalence of presbyopia is estimated to be 2 billion. An estimated 120 million Americans live with presbyopia, a large prevalence that is expected to exceed 150 million by 2034. To assist with their near vision deficiencies, individuals with presbyopia use reading glasses and contact lenses, and in some cases undergo surgical interventions. However, there are currently no approved drug therapies for presbyopia. As there are several drawbacks to reading glasses and contact lenses, including inconvenience, eye strain, and night vision disturbances, eye drops are increasingly being explored as an alternative. As for payment, presbyopia corrective devices have been mostly out-of-pocket cash pay by patients.

Marguerite McDonald, M.D., F.A.C.S, Clinical Professor of Ophthalmology at the NYU Langone Medical Center and Ocuphire Medical Advisory Board member, stated, “Presbyopia is a condition that affects nearly everyone over the age of 40. Many patients reliant on reading glasses and contact lenses desire more flexibility and convenience and continually

request alternatives or a complementary choice. Development of eyedrop treatments for Presbyopia has recently become very active, led by Allergan having recently submitted an NDA for this indication. There is room for many options given the large unmet need, and I am encouraged by Nyxol eye drops in combination with low-dose pilocarpine eye drops as a differentiated solution that works both on the iris dilator and sphincter muscles. This combination product should allow for moderate unopposed pupil constriction and provide the ability to see both at near and at distance without the side-effects such as brow ache, headache, blurry vision, and loss of distance night vision associated with the use of higher doses of pilocarpine."

Based on a GlobalData market research report, 69% of patients would consider an eye drop as an alternative to reading glasses. Importantly, over 70% of patients indicated that they wear reading glasses in the morning, afternoon, and evening, suggesting that patients desire a solution with durability. Over 50% of responders also indicated that using drops 2 to 4 times per day would be moderately to very convenient to them. In addition, 66% of surveyed eyecare providers indicated interest in a Nyxol and low-dose pilocarpine product profile as a potential therapeutic alternative to reading glasses.

About VEGA-1 Phase 2 Clinical Trial

The VEGA-1 clinical trial is designed to evaluate the efficacy and safety of Nyxol in combination with low-dose pilocarpine compared to placebo in presbyopic subjects. A total of 150 subjects (planned target was 140 to 152) have been enrolled at 17 investigational sites in the US from mid-February to mid-May this year. The Phase 2 trial is randomized, double-masked, placebo-controlled with 4 treatment arms. At the first visit, subjects are randomized to receive either Nyxol or placebo drops that are instilled at home near bedtime for 3 to 4 days prior to Visit 2; at Visit 2 subjects then receive either low-dose pilocarpine or no treatment with efficacy and safety measurements collected at multiple timepoints through 6 hours. The primary endpoint is the percentage of subjects with ≥ 15 letters of improvement in photopic binocular near vision (i.e. distance-corrected near visually acuity, DCNVA) at 1 hour on Visit 2 for Nyxol + low-dose pilocarpine arm compared to placebo alone arm. Secondary endpoints at multiple timepoints include improvements of 3 lines of DCNVA without any loss of distance vision, pupil diameter, and improvements of DCNVA at 1 and 2 lines compared to placebo as well as to each Nyxol and low-dose pilocarpine alone. Top-line results are expected by the end of Q2 2021.

Ocuphire collaborated closely with Oculos Development Services, a Tampa, Florida based clinical research organization and a subsidiary of Iuvo BioScience, on the launch and execution of the VEGA-1 trial. "We are pleased by the rapid enrollment in this Phase 2 trial, which speaks to the unmet need of an eye drop to benefit vision correction for patients with presbyopia. We thank our investigators, clinical coordinators, staff, and subjects for their support and ease of trial execution at 17 sites across the U.S.," said Chuck Slonim, MD, Chief Medical Officer and Medical Monitor of Oculos Development Services.

For more information about the VEGA-1 Phase 2 trial design and its U.S. clinical sites, please visit www.clinicaltrials.gov (NCT04675151).

About Ocuphire Pharma

Ocuphire is a publicly traded (NASDAQ: OCUP), clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of several eye disorders. Ocuphire's pipeline currently includes two small-molecule

product candidates targeting front and back of the eye indications. The company's lead product candidate, Nyxol® (0.75% phentolamine ophthalmic solution) Eye Drops, 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 dim light or night vision disturbances (NVD), reversal of pharmacologically-induced mydriasis (RM), and presbyopia, and has been studied in 8 clinical trials including the recently completed Phase 3 trial in RM. Ocuphire reported positive topline data in March 2021 for MIRA-2, a Phase 3 FDA registration study for treatment of RM. Nyxol is also currently in Phase 3 clinical development for NVD and in Phase 2 for presbyopia. 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) and has been studied in 11 Phase 1 and 2 trials. APX3330 is currently enrolling subjects in a Phase 2 clinical trial in subjects with DR/DME. As part of its strategy, Ocuphire will continue to explore opportunities to acquire additional ophthalmic assets and to seek strategic partners for late-stage development, regulatory preparation, and commercialization of drugs in key global markets. Please visit www.clinicaltrials.gov to learn more about Ocuphire's completed Phase 2 trials, recently completed Phase 3 registration trial (NCT04620213), ongoing Phase 3 registration trial (NCT04638660), Phase 2 trial in presbyopia (NCT04675151), and Phase 2 trial in DR/DME (NCT04692688). For more information, please 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 Ocuphire's product candidates, results of ongoing and future clinical trials, and commercialization and market opportunities. 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, and (ix) the success and timing of commercialization of any of Ocuphire's product candidates. 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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