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Ocuphire Pharma Announces First Patient Enrolled in VEGA-2 Pivotal Phase 3 Trial of Nyxol® in Presbyopia

Nyxol® Eye Drops Potentially Offer Differentiated Mechanism of Action to TreatAge-Related Blurry Near Vision

Launch of Presbyopia Phase 3 Program Supported by Positive Clinical Results that Demonstrated Durable Efficacy and Favorable Safety Profile

FARMINGTON HILLS, Mich., Jan. 09, 2023 (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 announced the initiation of the VEGA-2 Phase 3 pivotal trial with the first patient enrolled in late December. VEGA-2 is evaluating the efficacy and safety for two labels: Nyxol® alone and Nyxol with adjunctive low-dose pilocarpine (LDP) therapy for presbyopia.

“2023 promises to be a pivotal year for executing our comprehensive Phase 3 program in presbyopia,” said Mina Sooch, MBA, founder and CEO of Ocuphire. “We also recently achieved a major milestone with the submission of the NDA for the first Nyxol indication in reversal of mydriasis (RM). We are excited to add these important accomplishments to our recently closed global license agreement with FamyGen Life Sciences (recently acquired by Viatris [Nasdaq: VTRS]), for the development and commercialization of Nyxol across three indications.”

Jay Pepose, MD, PhD, Chief Medical Advisor of Ocuphire, stated, “In our previous Phase 2 VEGA-1 trial, Nyxol alone demonstrated compelling results with rapid onset and sustained 18-hour duration of efficacy and a favorable safety profile; then the LDP combination option also offers the potential for tunability of treatment based on the patient’s lifestyle and response to Nyxol alone. Physicians are enthusiastic about the potential for a safe, effective, convenient and durable pharmacologic option for their presbyopia patients.”

VEGA-2 Phase 3 Pivotal Trial Design

VEGA-2 is a randomized, double-masked, placebo-controlled, multi-center, Phase 3 study to evaluate Nyxol (phentolamine ophthalmic solution 0.75%) as a single agent and with adjunctive low-dose pilocarpine (LDP) 0.4% in 320 subjects with presbyopia. The study will be conducted in 2 stages. Stage 1 has two treatment groups (Nyxol or placebo), with approximately 160 subjects in each group. Stage 2 will have four treatment groups (Nyxol + LDP, Nyxol + LDP vehicle, placebo + LDP, and placebo + LDP vehicle), with approximately

80 subjects per treatment group. Subjects will be recruited from upto 30 investigational sites in the US. For more information on the trial design and endpoints, please refer to www.ClinicalTrials.gov Identifier NCT05646719.

In addition to VEGA-2 trial, Ocuphire plans to initiate a second Phase 3 pivotal trial in presbyopia (VEGA-3), and a one-year safety study (LYRA-1) in 2023.

About Presbyopia

Presbyopia is characterized as age-related blurred near vision, with onset most common in people over 40 years old. As the eye ages, the lens becomes stiffer, which limits the eye's ability to adjust its focus for reading or for other tasks that require clear vision at near distances. Presbyopia patients experience blurred near vision, difficulty seeing in dim light, and eye strain. Because of the ubiquity of the condition, presbyopia represents a large market both in the United States and overseas totaling over 2 billion presbyopia patients. It is estimated that 128 million Americans have presbyopia, and this number is expected to grow as the population above the age of 45 increases.

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 has a partnership with FamyGen Life Sciences and Viatris 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 uniquely modulate the pupil size by blocking the α1 receptors found only on the iris dilator muscle without affecting the ciliary muscle. Nyxol is being evaluated for three potential indications, including single-use for reversal of pharmacologically-induced mydriasis (RM), and once-daily for treatment of presbyopia and dim light or night vision disturbances (NVD). Nyxol has been studied in a total of 12 clinical trials (3 Phase 1, 5 Phase 2, 4 Phase 3) in a total of approximately 1,100 patients (with over 650 Nyxol-treated) and has demonstrated promising clinical data for use in the multiple ophthalmic indications. Ocuphire reported positive top-line data from the Phase 3 MIRA-2, MIRA-3 and MIRA-4 trials in RM, Phase 3 LYNX-1 trial in NVD and Phase 2b VEGA-1 trial for Nyxol as single agent and as adjunctive therapy with 0.4% low dose pilocarpine in presbyopia. Nyxol has an NDA submission for RM under the 505(b)(2) pathway, and currently in Phase 3 for presbyopia and NVD.

The Company's late-stage 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). DR affects over 8 million diabetics in the US and is a large unmet need with limited treatment options. APX3330 has been studied in 11 Phase 1 and 2 trials. The Company announced the completion of last patient last visit in late August with top-line results expected in early 2023 (NCT04692688).

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 initiation of clinical trials, receipt of data from clinical trials, submission and receipt of regulatory approvals, Ocuphire's business strategy and potential growth, and timelines. 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 partnerships with Famy and Viatris 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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