September 5, 2024

Ocuphire Pharma Initiates VEGA-3 Phase 3 Trial Evaluating Phentolamine Ophthalmic Solution 0.75% for Presbyopia

Top-line data from VEGA-3 Phase 3 trial expected in first half of 2025

Top-line data from LYNX-2 Phase 3 trial in patients with decreased visual acuity under low light conditions following keratorefractive surgery expected in first quarter of 2025

FARMINGTON HILLS, Mich., Sept. 05, 2024 (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 patients with retinal and refractive eye disorders, today announced that the VEGA-3 Phase 3 clinical trial evaluating Phentolamine Ophthalmic Solution 0.75% for presbyopia has dosed its first participants.

Presbyopia, the gradual loss of ability to focus on near objects, typically becomes noticeable in the early to mid-40s. This progressive and ubiquitous condition leads to the widespread use of reading glasses or bifocals. Phentolamine Ophthalmic Solution 0.75% is being developed to provide a non-invasive, convenient alternative to traditional corrective measures.

“Our goal is to provide a safe, long-lasting, effective solution that restores near vision and enhances overall visual performance in people with presbyopia, under both daytime and nighttime conditions,” said George Magrath, M.D., M.B.A., M.S., Ocuphire’s Chief Executive Officer. “We are pleased to begin the VEGA-3 trial, building on the positive results generated in our prior presbyopia studies, which have shown a rapid onset of action, favorable safety profile, and sustained duration of effect that are promising at this stage.”

Ocuphire anticipates using data obtained from the VEGA-3 trial demonstrating the efficacy and safety of Phentolamine Ophthalmic Solution 0.75% as a potential treatment for presbyopia to support a supplemental New Drug Application with the U.S. Food and Drug Administration (the “FDA”).

Dr. Magrat continued, “We are also excited about the continued enrollment in the LYNX-2 trial. Our top-line data for LYNX-2 are expected in the first quarter of 2025, assuming enrollment continues at the current rate. I’m very proud of the work our team and partners have put into the execution of this study.”

VEGA-3 Phase 3 Pivotal Trial Design

VEGA-3 is a randomized, double-masked, placebo-controlled, multi-center, Phase 3 clinical

trial evaluating Phentolamine Ophthalmic Solution 0.75% in 545 participants with presbyopia. Participants are randomized 3:2 to receive one drop of Phentolamine Ophthalmic Solution 0.75% or placebo each evening. The primary endpoint is the percentage of participants with 15-letter improvement in photopic binocular distance-corrected near visual acuity ("DCNVA") on the eighth day following their first visit. The improvement in binocular DCNVA for each participant will be relative to their own baseline value. Participants will be followed a total of 48 weeks to collect chronic safety data. Recruitment will take place at up to 40 investigational sites in the U.S. For more information on the trial design and endpoints, please refer to www.ClinicalTrials.gov (NCT06542497).

Top Line Data from LYNX-2 Phase 3 Study Expected Q1 2025

The LYNX-2 Phase 3 trial is a randomized, double-masked, placebo-controlled Phase 3 registration trial designed to evaluate the safety and efficacy of Phentolamine Ophthalmic Solution 0.75% compared to placebo in participants who underwent keratorefractive surgery and then reported glare, halos or starbursts and demonstrated low contrast visual acuity under mesopic (low) light conditions. The LYNX-2 trial is being conducted under conditions of a Special Protocol Assessment (SPA) with the FDA. The trial is expected to enroll 200 participants. The primary endpoint, agreed with the FDA under the SPA, will be a gain of 3 lines (or 15 letters) or more of distance vision improvement on a low contrast chart in low light conditions after 15 days of dosing. Top line data are expected in the first quarter of 2025. Additional information about the LYNX-2 Phase 3 trial can be found at www.ClinicalTrials.gov (NCT06349759).

There are currently no FDA-approved treatments for visual disturbances under low light conditions. With a mechanism of action that moderately reduces pupil size without the increased risks of retinal tears or detachment associated with parasympathomimetic miotics that engage the ciliary muscle, Phentolamine Ophthalmic Solution 0.75% eye drops have the potential to be a treatment option that could improve patients' ability to see and function in low light following keratorefractive surgery.

Ocuphire is responsible for managing the VEGA-3 and LYNX-2 trials. Under the terms of the License Agreement, Ocuphire's partner will reimburse Ocuphire for agreed-to budgeted costs related to the development of Phentolamine Ophthalmic Solution 0.75% through FDA approval, and then share costs above an agreed upon threshold amount.

About Phentolamine Ophthalmic Solution 0.75%

Phentolamine Ophthalmic Solution 0.75%, Ocuphire's late-stage product candidate, is a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size. It works by uniquely blocking the alpha-1 receptors found on the radial iris dilator muscles, which are activated by the alpha-1 adrenergic receptors, without affecting the ciliary muscle. Phentolamine Ophthalmic Solution 0.75% is being developed for presbyopia and dim (mesopic) light vision disturbances (sometimes referred to as DLD) after keratorefractive surgery. Phase 2 and Phase 3 trials for the use of Phentolamine Ophthalmic Solution 0.75% to treat presbyopia have met their primary endpoints. It is currently approved and marketed by our partner as RYZUMVI™ for the treatment of pharmacologically induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents.

About Presbyopia

As the eye ages, the ability to focus for reading and other tasks that require clear vision at near distances decreases. Presbyopia patients experience blurred near vision, difficulty seeing in dim light and eye strain. It is estimated that 128 million Americans, and over 2 billion people worldwide, have presbyopia, and this number is expected to grow as the population ages.

About Dim Light Disturbances after Keratorefractive Surgery

Decreased visual acuity under low light conditions ("DLD") is characterized by peripheral corneal imperfections (aberrations) that result in unfocused light when the pupil dilates under low light conditions. Patients with DLD experience decreased low contrast visual acuity as well as glare, halos, and starbursts.

About Ocuphire Pharma

Ocuphire Pharma, Inc. (Nasdaq: OCUP) is a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing novel therapies for the treatment of patients with retinal and refractive eye disorders. Ocuphire's lead product candidate, APX3330, a novel small-molecule inhibitor of Ref-1 (reduction oxidation effector factor-1 protein), is in development for diabetic retinopathy. In addition, Ocuphire's late-stage product candidate Phentolamine Ophthalmic Solution 0.75%, a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size, is being developed for presbyopia and DLD and is currently approved and marketed by our partner as RYZUMVI™ for the treatment of pharmacologically induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents. For more information, please visit www.ocuphire.com.

Forward Looking Statements

This press release contains 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 efficacy and safety of Phentolamine Ophthalmic Solution 0.75% in the treatment of presbyopia and dim (mesopic) light vision disturbances, logistical details regarding the VEGA-3 Phase 3 clinical trial, our plan to submit a New Drug Application based on the results of the upcoming VEGA-3 Phase 3 clinical trial, expectations about when data will be available from the LYNX-2 Phase 3 trial, and continued drug development and marketing under our partnership agreement.

These forward-looking statements relate to us, our business prospects and our results of operations and are subject to certain risks and uncertainties posed by many factors and events that could cause our actual business, prospects and results of operations to differ materially from those anticipated by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those described under the heading "Risk Factors" included in our Annual Report on Form 10-K. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. In some cases, you can identify forward-looking statements by the following words: "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "aim," "may," "ongoing," "plan," "potential," "predict," "project," "should," "will,"

“would” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. We undertake no obligation to revise any forward-looking statements in order to reflect events or circumstances that might subsequently arise.

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:

- The success and timing of regulatory submissions and pre-clinical and clinical trials, including enrollment and data readouts;
- Regulatory requirements or developments;
- Changes to or unanticipated events in connection with clinical trial designs and regulatory pathways;
- Delays or difficulties in the enrollment of patients in clinical trials;
- Substantial competition and rapid technological change;
- Our development of sales and marketing infrastructure;
- Future revenue losses and profitability;
- Our relatively short operating history;
- Changes in capital resource requirements;
- Risks related to the inability of Ocuphire to obtain sufficient additional capital to continue to advance its product candidates and its preclinical programs;
- Domestic and worldwide legislative, regulatory, political and economic developments;
- Employee misconduct;
- Changes in market opportunities and acceptance;
- Reliance on third parties;
- Future, potential product liability and securities litigation;
- System failures, unplanned events, or cyber incidents;
- The substantial number of shares subject to potential issuance associated with our equity line of credit arrangement;
- Risks that our partnership or other licensing arrangements, may not facilitate the commercialization or market acceptance of Ocuphire’s product candidates;

- Future fluctuations in the market price of our common stock;
- The success and timing of commercialization of any of Ocuphire's product candidates; and
- Obtaining and maintaining 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. Readers are urged to carefully review and consider the various disclosures made by us in this report and in our other reports filed with the Securities and Exchange Commission that advise interested parties of the risks and factors that may affect our business. 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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Source: Ocuphire Pharma