June 30, 2021

Ocuphire's VEGA-1 Phase 2 Trial in Presbyopia Meets Primary and Secondary Endpoints

Met primary endpoint with statistical significance at 1 hour with 61% of subjects treated with Nyxol® plus low-dose pilocarpine (LDP) gaining ≥ 15 letters (3 lines) in near vision

Key secondary endpoints on visual acuity and pupil diameter showed statistical significance

Nyxol plus LDP showed a favorable safety profile

Plans to advance into Phase 3 registration trials

Conference call and live webcast @ 8.30 am ET today

FARMINGTON HILLS, Mich., June 30, 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, today announced that the VEGA-1 Phase 2 clinical trial evaluating the efficacy and safety of Nyxol in combination with low-dose pilocarpine (LDP) in presbyopic subjects successfully met its primary and many secondary endpoints. On the strength of these results, Ocuphire plans to move into Phase 3. Given the rapid onset and sustained duration of efficacy, the favorable safety profile, and the potential tunability of treatment, Nyxol + LDP has the potential for differentiation and to be a best in class product for the treatment of presbyopia.

Highlights from the VEGA-1 Phase 2 Trial in Presbyopia:

Nyxol + LDP Met the Primary Endpoint

- 61% of subjects treated with Nyxol + LDP improved 15 letters or greater (≥ 3 lines) in photopic binocular near vision at 1 hour compared with 28% of subjects on placebo with statistical significance (p = 0.003 with placebo adjusted difference of 33%)

Nyxol + LDP Met Many Additional Efficacy Endpoints

- Met the Phase 3 co-primary endpoint vs. placebo gaining 15 letters (3 lines) near vision with less than 5 letters of distance vision loss
- Rapid onset of efficacy at 30 mins
- Durable near vision improvement through at least 6 hours
- Sustained significant reduction in pupil diameter over at least 18 hours due to the durable effects of Nyxol
- Near vision efficacy seen both monocularly and binocularly

- Efficacy in both light and dark iris colors

Nyxol + LDP Showed a Favorable Safety Profile

- No serious AEs, almost all AEs were mild
- No headaches, no brow aches, and no blurry vision AEs were reported
- Mild, transient conjunctival hyperemia (eye redness) observed in <5% of subjects

Jay S. Pepose, MD, PhD, Director of the Pepose Vision Institute, Professor of Clinical Ophthalmology at the Washington University School of Medicine, and Ocuphire Medical Advisory Board and Corporate Board member, commented, “The results from this Phase 2 VEGA-1 trial validate Nyxol’s mechanism of action on iris dilator muscle and the beneficial effects of smaller pupil size in treating presbyopia. These latest data support a clinical profile for Nyxol plus LDP combination that includes rapid onset of action and sustained duration of effect, while maintaining distance visual acuity in day and night conditions. All treatments were well tolerated and demonstrated a favorable safety profile. Taken together, we believe these attributes position Nyxol + LDP as a potential ‘best in class’ presbyopia treatment option.”

Presbyopia is a gradual, age-related loss of the eyes’ ability to focus on nearby objects. The global prevalence is estimated to be 2 billion. Approximately 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 in the United States. 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 treatment modality.

Susan Benton of Ocuphire’s Board of Directors remarked, “The need for an eyedrop treatment is highlighted by industry leader Allergan and several other companies developing pharmacological treatment options for presbyopia. Ocuphire’s novel target product profile of a combination kit of Nyxol and LDP may offer rapid onset and long-lasting effects with ‘tunability’ as an option in that all patients are not the same (one size does not fit all). A combination kit option may provide a "range" of pupillary modulation that the doctor can customize to the patient to optimize their near vision. This ability to customize therapy will be more difficult for fixed-dose combinations and single-agent products.”

“We are thrilled with the positive outcome in VEGA-1, which showed that a combination of Nyxol and low-dose pilocarpine produced a statistically significant improvement in near visual acuity in subjects with presbyopia,” said Mina Sooch, MBA, President and CEO of Ocuphire Pharma. “We would like to thank all of the subjects and investigational sites that participated in our first presbyopia clinical trial for Nyxol. Presbyopia represents an area of considerable unmet need due to its rising prevalence worldwide and the limitations of currently available corrective methods. Based on the data generated thus far, we believe that Nyxol and LDP is novel in its mechanism of action and could become a leading pharmacological treatment option for presbyopia and potentially allow those afflicted to reduce their dependence on reading glasses. We plan to initiate our Phase 3 trials for presbyopia in 2022, building on our recent success of Nyxol for Reversal of Mydriasis with initiation of the second Phase 3 registration trial later this year.”

VEGA-1 Phase 2 Trial Design
The VEGA-1 Phase 2 clinical trial was 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) were enrolled at 17 investigational sites in the US from mid-February to mid-May of this year. The Phase 2 trial was a randomized, double-masked, placebo-controlled study with 4 treatment arms. At the first visit, subjects were randomized to receive either Nyxol or placebo drops that were instilled at home near bedtime for 3 to 4 days prior to Visit 2; at Visit 2 subjects then received either low-dose pilocarpine or no treatment, with efficacy and safety measurements collected at multiple timepoints through 6 hours. The primary endpoint was 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 + LDP arm compared to placebo alone arm. The study was powered for comparison to placebo whereas comparison to component arms were designed to inform the Phase 3 sample size for a combination product approval. Secondary endpoints at multiple timepoints included Nyxol + LDP improvements of 3 lines of DCNVA without any loss of distance vision, pupil diameter, and improvements of DCNVA of 1 and 2 lines compared to placebo as well as to Nyxol and low-dose pilocarpine alone. For more information, refer to ClinicalTrials.gov Identifier: NCT04675151.

Ocuphire collaborated closely with Oculos Development Services, a Rush, NY based clinical research organization and a subsidiary of iuvo BioScience, on the launch and execution of the VEGA-1 trial.

Detailed results of the VEGA-1 study will be presented by Dr. Pepose at the upcoming American Society of Cataract and Refractive Surgery (ASCRS) medical meeting: *VEGA-1 Presbyopia Presentation on Sunday July 25, 2021 at 8:45am (ASCRS Paper ID 76645).*

Conference Call and Webcast (with slides)
Ocuphire management will host a conference call and webcast with slides, today at 8.30am ET. Details for the call are as follows:

Toll free (U.S.)	877-407-4018
International:	201-689-8471
Conference ID	13721064
Webcast:	http://public.viavid.com/index.php?id=145478

The webcast will also be available on the “Investors” tab of the Ocuphire corporate website tab, under News & Events and will be archived for 90 days.

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 9 clinical trials including the recently completed Phase 3 trial in RM and Phase 2 trial in presbyopia. 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. 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 in RM (NCT04620213), ongoing Phase 3 registration trial in NVD (NCT04638660), recently completed 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 a potential Phase 3 trial in presbyopia, Nyxol + LDP's potential to be a 'best in class' presbyopia treatment option, and the market and commercial potential of Nyxol + LDP. 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.

Ocuphire Contacts

Mina Sooch, President & CEO
Ocuphire Pharma, Inc.
ir@ocuphire.com
www.ocuphire.com

Corey Davis, Ph.D.
LifeSci Advisors
cdavis@lifesciadvisors.com

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