February 18, 2021

Ocuphire Initiates Enrollment in VEGA-1 Phase 2 Trial Investigating Nyxol in Presbyopia

Nyxol and Low-Dose (0.4%) Pilocarpine Combination to Target Improvement in Near Vision in a Presbyopic Population

Top Line Data from VEGA-1 Expected End of 2Q21

FARMINGTON HILLS, Mich., Feb. 18, 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 enrolled the first patients in a Phase 2 proof of concept trial, titled VEGA-1, to evaluate a combination kit of Nyxol and low-dose pilocarpine in presbyopia. Over 12 sites representing a majority of the planned clinical sites are activated and recruiting patients.

Nyxol's potential to improve near vision is based on its mechanism of reducing pupil diameter which results in an increased depth of focus. Nyxol alone in multiple Phase 2 trials has reduced pupil diameter by approximately 20% and has significantly improved near visual acuity by one eye-chart line for over 24 hours after an evening eye drop. It has been well established by the field that reducing pupil diameter to the 1.6 to 2.0 mm range ("pinhole effect") can lead to significant improvements in near vision for presbyopic patients. Nyxol is being evaluated as part of a two-drug kit provided to patients, with Nyxol to be applied once daily in the evening and low-dose pilocarpine to be dosed in the daytime. According to a market research report by GlobalData, 40% of patients would request an alternative to reading glasses if available and 69% of patients would consider an eye drop alternative. Some key factors that will influence patient adoption will be efficacy, tolerability, comfort, onset of action, durability, and good distance vision.

Mina Sooch, MBA, President and CEO of Ocuphire Pharma commented, "Presbyopia could be one of the largest patient populations at over 100 million to be treated with a pharmacologic option. Many people with presbyopia may greatly benefit from an eye drop treatment as an alternative to reading glasses. We believe that the combination of Nyxol and low-dose pilocarpine has an ideal product profile to become a promising treatment for presbyopia. We are pleased to begin enrollment in the VEGA-1 clinical trial working with our CRO partner Oculos and look forward to reporting results at the end of the second quarter this year. This is our 3rd later stage clinical trial launched for Nyxol in just the last few months."

"I am very happy to see that the VEGA-1 trial has begun enrollment, which brings the

potential for Nyxol and low-dose pilocarpine eye drops to allow presbyopia patients to experience the ability to see both at near and at distance without the dependence on reading glasses. The combination of Nyxol moderately inhibiting the iris dilator muscle and low dose of pilocarpine, an extensively studied miotic, moderately activating the iris constrictor muscle should allow for unopposed pupil constriction to reach the 'pin-hole'. Ocuphire's use of agents working on both iris muscles to manage pupil size is a key differentiating factor for its approach," says Dr. Marguerite McDonald, Clinical Professor of Ophthalmology at the NYU Langone Medical Center.

VEGA-1 is a double-masked, randomized, placebo-controlled, multi-center trial designed to evaluate 0.75% Nyxol in combination with low dose (0.4%) pilocarpine for treatment of presbyopia. The trial is expected to enroll approximately 152 patients with a clinical diagnosis of presbyopia (near visual acuity of 20/50 or worse). The primary endpoint is percentage of patients with at least 3 lines (15 letters or more) of binocular distance corrected near visual acuity (DCNVA) improvement on a standard near vision eye chart in daytime (photopic) lighting conditions. Secondary endpoints at multiple timepoints include improvements in 3 lines of DCNVA without any loss of distance vision, pupil diameter, and improvements in DCNVA at 1 and 2 lines compared to placebo as well as to each Nyxol and low-dose pilocarpine alone. For more information refer to ClinicalTrials.gov Identifier: NCT04675151.

About Presbyopia

Presbyopia is an age-related condition 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. It is estimated that 120 million Americans have presbyopia and this number is expected to grow as the population above the age of 45 increases. Currently, there are no pharmacological therapies approved for presbyopia, but there is evidence that decreasing pupil diameter, especially to a size of 1.6 mm to 2.0 mm to create a "pinhole" effect, can improve near visual acuity by increasing the depth of focus.

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® 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 7 Phase 1 and 2 trials. 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. Nyxol is entering Phase 3 clinical development for NVD and RM, and Phase 2 for presbyopia. APX3330 is entering Phase 2 clinical development for 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 clinical trials and ongoing Phase 3 registration trials (NCT04620213 and NCT04638660), Phase 2 trial in presbyopia (NCT04675151) and soon to recruit 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; (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.

Ocuphire Contacts

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

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

Source: Ocuphire Pharma