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# Ocuphire Completes Enrollment in MIRA-2 Phase 3 Clinical Trial Investigating Nyxol® for Reversal of Mydriasis

*Nyxol has Potential to be a New Treatment Option for Reversal of Pharmacologically-Induced Pupil Dilation*

*Top-Line Data for MIRA-2 Expected by End of Q1 2021*

FARMINGTON HILLS, Mich., Jan. 06, 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 earlier than expected in its MIRA-2 (NCT04620213) Phase 3 registration clinical trial evaluating the safety and efficacy of Nyxol® to reverse pharmacologically-induced mydriasis.

“We are pleased to exceed our enrollment target and reach this important milestone in our Reversal of Mydriasis MIRA-2 program in just over 4 weeks despite the challenges presented by the COVID-19 pandemic,” said Mina Sooch, MBA, President and CEO of Ocuphire Pharma. “This current study builds on the positive results achieved in our recent Phase 2b MIRA-1 trial, which provided evidence of Nyxol’s ability to more rapidly return pupil diameter back to normal baselines. MIRA-2 is the first completed trial of our four planned Phase 2 and 3 trials for Nyxol and APX3330 in the upcoming year, highlighting management’s ability to execute on our strategic plan.”

Every year in the U.S., approximately 100 million eye exams are performed that require dilation of the pupil (mydriasis) to examine the back of the eye either for routine check-ups, disease monitoring or surgical procedures. Depending on the individual and the color of their eyes, the pharmacologically-induced dilation can last anywhere from 6 to 24 hours. Dilated eyes have a heightened sensitivity to light and an inability to focus on near objects, causing difficulty with reading, working, and driving.

Ocuphire has been working closely with the market research firm GlobalData to survey several hundred patients and eye care providers (optometrists and ophthalmologists) about Reversal of Mydriasis (as well as Night Vision Disturbances and Presbyopia). Over 65% of surveyed patients reported moderate to severe negative impact of a dilated exam, underscoring the potential value of Nyxol’s role in improving comfort and daily function after pupil dilation. Additionally, an estimated 45% of patients responded that they would be very likely to request a dilation reversal drop, and more than 40% of eye care providers would be likely to use a reversal drop if such a treatment were commercially available.

Nyxol is a proprietary, preservative-free, stable eye drop formulation of phentolamine mesylate that reduces pupil size through acting on the iris dilator muscle. It has been studied in seven Phase 1 and 2 trials that have characterized Nyxol's efficacy, durability, and tolerability product profile. One of these trials was MIRA-1, a randomized cross-over, double-masked, placebo-controlled, multi-center Phase 2 trial studying Nyxol in reversal of mydriasis. The results showed with statistical significance that, following pharmacologically-induced dilation, pupils treated with Nyxol returned to their normal size more rapidly than those with placebo. Highlights from this trial were presented by Dr. Paul Karpecki at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting 2020. Ocuphire is also pleased to announce the acceptance of a peer-reviewed written publication of the MIRA-1 study "Phentolamine Eye Drops Reverse Pharmacologically Induced Mydriasis in a Randomized Phase 2b Trial" in Optometry and Vision Science (OVS), Journal of the American Academy of Optometry. The article will appear soon in print and on-line at www.optvissci.com.

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 member commented, "Patients who have had a routine eye exam and request some form of dilation reversal agent currently have no commercially available option and may find driving home with blurry vision challenging. The MIRA-2 Phase 3 clinical trial is designed to confirm Nyxol's consistent and clinically relevant reduction in pupil diameter and overall safety profile demonstrated in earlier registered trials and to support the product's potential commercialization and adoption in eye care practices."

MIRA-2 trial is designed to evaluate the efficacy and safety of Nyxol compared to placebo in healthy subjects who had received mydriatic (dilating) drops. A total of 185 healthy pediatric and adult subjects (target of 168) were enrolled into this 24-hour, multi-center, randomized, double-masked, placebo-controlled Phase 3 trial. At the treatment visit, subjects who have been randomized and also stratified by iris color are administered one of three approved mydriatic agents (phenylephrine, tropicamide, or Paremyd®) approximately 1 hour prior to receiving study treatment with either Nyxol or placebo. The primary endpoint is the percentage of subjects returning to their baseline pupil diameter within 90 minutes. Top-line results are expected by the end of Q1 2021. MIRA-2 is the first of two registration trials planned for Nyxol in this acute indication prior to submission of a New Drug Application.

Ocuphire is collaborating closely with Oculos Development Services, a Tampa, Florida based clinical research organization and subsidiary of Iuvo BioScience, on the launch and execution of the MIRA-2 trial. "We are pleased by the rapid enrollment in this Phase 3 trial, which speaks to the unmet need. We thank our investigators, clinical coordinators, staff, and subjects for their support and ease of trial execution at 12 sites across the U.S.," said Chuck Slonim, MD, Chief Medical Officer and Medical Monitor of Oculos.

For more information about the MIRA-2 Phase 3 trial design and its U.S. clinical sites, please visit www.clinicaltrials.gov (NCT04620213).

**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 pharmacologically-induced mydriasis (RM), and 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). 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). 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 and potential. 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, and (vii) the effects of COVID-19 on clinical programs and business operations. 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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