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Ocuphire Completes Enrollment in MIRA-4 Pediatric Safety Trial Evaluating Nyxol® for Reversal of Mydriasis

With Growing Rate of Childhood Myopia, MIRA-4 Trial Supports a Potential Broader RM Label for Nyxol in Pediatric Subjects 3 to 11 Years

Ocuphire Delivering on Execution Early in 2022, Momentum Builds with Completion of Patient Enrollment in 3 Late-Stage Trials for Nyxol

Upcoming Top-Line Data for Pivotal MIRA-3 Trial Expected End of Q1 2022

FARMINGTON HILLS, Mich., March 08, 2022 (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 that it has completed enrollment in the MIRA-4 trial evaluating the safety and efficacy of Nyxol® eye drops to reverse pharmacologically-induced mydriasis (RM) in 23 pediatric subjects. Ocuphire worked closely with Oculos Development Services and the 2 U.S. investigational sites to accomplish this milestone in 2 months.

MIRA-4 is part of a comprehensive MIRA clinical program to develop Nyxol for RM and as agreed with FDA under the Pediatric Research Equity Act. This includes the evaluation in pediatric patients as young as 3 to 11 years old in order to include Nyxol use for this pediatric population. In February, Ocuphire announced completion of enrollment in the MIRA-3 Phase 3 clinical trial investigating Nyxol for RM in adults 18 and older and children of ages 12-17 years. If successful, the company anticipates submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for Nyxol for RM in late 2022. With no current commercially available therapies, Nyxol has the potential to address an estimated $500 million reversal of dilation market including pediatric and adult populations where adrenergic (phenylephrine) and parasympatholytic (tropicamide, cyclopentolate, etc.) agents are commonly used for dilating eyes. These classes of dilating drops were studied in the MIRA program.

“Due to the myopia epidemic which is expected to affect 50% of world’s populations by 2050, we expect an increase in refractions and dilations in children, especially in Asia where high myopia is so prevalent,” stated Jay Pepose, MD, Ph.D. Medical Advisor, Ocuphire Pharma. “The effects of dilations can impact multiple stakeholders including children, their parents and even teachers, facilitating the child’s return to school, sports and homework. Nyxol may allow parents and physicians to explore a new treatment option to reverse mydriasis.”

Mina Sooch, MBA, CEO and Founder, Ocuphire Pharma commented, “We are building

momentum in the first months of 2022 as we report rapid enrollment and completion in three of our late stage trials for Nyxol. If MIRA-4 meets its endpoints, the results would potentially support a broader label for Nyxol in RM to include children as young as age 3. MIRA-3 and MIRA-4 will build upon positive results in the MIRA program and complete the clinical data package for our planned NDA submission to FDA later this year. We expect top-line results from MIRA-3 RM Phase 3 study by end of 1Q22, followed by the LYNX-1 NVD Phase 3 study and MIRA-4 RM pediatric study in 2Q22."

MIRA 4 Study Design

MIRA-4 is a multi-center, randomized, parallel-arm, double-masked, placebo-controlled study targeting 20 randomized pediatric subjects (10 in ages 3 to 5, and 10 in ages 6 to 11) evaluating the safety and efficacy of Nyxol in pharmacologically-induced mydriasis with three dilating agents (phenylephrine, tropicamide, and Paremyd). The primary endpoint is safety with descriptive statistics for secondary efficacy endpoints. For more information, please see NCT05223478.

Reversal of Mydriasis Market Opportunity: Adults and Pediatrics

An estimated 100 million eye dilations are conducted every year in the U.S. to examine the back of the eye either for routine check-ups, disease monitoring or surgical procedures across all eye care practice groups. Depending on the individual and the color of their eyes, the pharmacologically-induced dilation can last anywhere from 6 to 24 hours in adults. Dilated eyes have heightened sensitivity to light and an inability to focus on near objects, causing difficulty reading, working, and driving. Currently, there are no approved or available treatment options to safely reverse mydriasis. If approved, Nyxol has the potential to be the first and only FDA-approved agent for the reversal of mydriasis uniquely modulating the iris dilator muscle.

According to the American Association for Pediatric Ophthalmology and Strabismus, dilating eye drops can last anywhere from 4 to 24 hours in children depending on the strength, type of the drop and the individual patient.

Market research conducted by GlobalData surveyed several hundred patients and eye care providers (optometrists and ophthalmologists) about reversal of mydriasis. Over 65% of surveyed patients reported moderate to severe negative impact of a dilated pupil. These data underscore the potential value of the role of the product candidate Nyxol in improving comfort and daily function after pupil dilation. Furthermore, approximately 80% of patients responded that they would be likely to request a dilation reversal drop, and more than 70% of eye care providers would be likely to use a reversal drop. The market research confirmed patients' willingness to pay out-of-pocket to reverse their dilations, supporting a market size estimate of over $500M. Ocuphire is currently evaluating partnering options for an effective and cost-efficient commercial launch of Nyxol targeted for the second half of 2023, if approved.

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 refractive and retinal indications. The company's lead product candidate, Nyxol® eye drops (0.75% phentolamine ophthalmic solution) 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 reversal of pharmacologically-induced mydriasis (RM), presbyopia and dim light or night vision disturbances (NVD), and has been studied in 9 completed clinical trials through the end of 2021. Ocuphire reported positive top-line data for MIRA-2, the first Phase 3 registration trial for treatment of RM, and recently initiated and completed enrollment in the second Phase 3 registration trial (MIRA-3) and pediatric safety trial in RM. Ocuphire also reported positive top-line data from a Phase 2 trial of Nyxol for treatment of presbyopia, both alone and with low dose pilocarpine ("LDP") 0.4% as adjunctive therapy. The company recently completed enrollment in its Phase 3 study of Nyxol 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. The company is currently enrolling subjects in a Phase 2b clinical trial of APX3330 to treat DR/DME (ZETA-1). 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 recently enrolled second Phase 3 registration trial in RM (NCT05134974), MIRA-4 pediatric safety study in RM (NCT05223478), and Phase 3 registration trial in NVD (NCT04638660) and actively enrolling Phase 2b trial in DR/DME (NCT04692688). Ocuphire previously completed the first Phase 3 registration trial in RM (NCT04620213), Phase 2 trial in presbyopia (NCT04675151). 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 regulatory timelines, commercial timelines, and future clinical trials in RM, presbyopia, NVD and DR/DME. 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.

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