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Ocuphire Announces FDA Acceptance of New Drug Application and PDUFA Date of September 28, 2023 for Nyxol® Eye Drops for Reversal of Mydriasis

If Approved Later This Year, Nyxol Could be the Only Commercially Available Eye Drop for Reversal of Dilation

FARMINGTON HILLS, Mich., Feb. 13, 2023 (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 the U.S. Food and Drug Administration (FDA) has accepted the New Drug Application (NDA) for Nyxol® (phentolamine ophthalmic solution 0.75%) for the treatment of pharmacologically-induced mydriasis (RM). The FDA assigned a Prescription Drug User Fee Act (PDUFA) date of September 28, 2023.

“The FDA’s acceptance of the NDA submission and PDUFA date in late September for Nyxol sets the stage for an exciting 2023”, said Mina Sooch, MBA, founder and CEO of Ocuphire Pharma. “We look forward to working closely with the FDA during the review process. If approved later this year, Nyxol eye drops will be the first prescription reversal drop available for patients in the US. In addition, we look forward to leveraging the synergies of this first NDA for Nyxol in potential supplementary NDAs for presbyopia and dim light vision disturbances indications in the future.”

Dr. Paul Karpecki, Clinical Director of Cornea at Kentucky Eye Institute, stated “Over 100 million eye dilations are performed each year in the US in pediatric and adult patients, and we expect dilations, which are the standard of care for comprehensive eye exams, to increase due to an aging population. Patients often express frustration and discomfort with the effects of prolonged dilation and eye care professionals underestimate the time it takes for patients to return to normal pupil size. A reversal drop would significantly improve patient experience and productivity.”

The NDA filing is supported by positive results from the comprehensive MIRA clinical program collectively involving over 600 subjects, including the MIRA-1 Phase 2b trial, MIRA-2 and MIRA-3 Phase 3 pivotal trials, and MIRA-4 Phase 3 pediatric trial. The MIRA-2 and MIRA-3 trials successfully met their primary and key secondary endpoints, demonstrating statistically significant superiority of Nyxol compared to placebo to rapidly return dilated eyes to their baseline pupil diameter as early as 60 and 90 minutes. Nyxol consistently showed a favorable safety and tolerability profile across all trials. In addition, the positive MIRA-4 pediatric trial results support a potential broader label for Nyxol in RM to include subjects aged 3 and older.

About Nyxol (Phentolamine Eye Drops)

Nyxol is a proprietary, preservative-free, stable, investigational eye drop formulation of phentolamine ophthalmic solution 0.75% designed to uniquely modulate the pupil size by blocking the α_1 receptors found only on the iris dilator muscle without affecting the ciliary muscle. Nyxol is being developed for reversal of pharmacologically-induced mydriasis (RM), presbyopia, and dim light (night) vision disturbances (DLD) under the 505(b)(2) pathway. Nyxol has been studied in a total of 12 clinical trials (3 Phase 1, 5 Phase 2, 4 Phase 3) in a total of approximately 1,100 patients (with over 650 Nyxol-treated) and has demonstrated promising clinical data for use in the multiple ophthalmic indications mentioned above. Ocuphire reported positive efficacy data from the MIRA-1, MIRA-2, MIRA-3 and MIRA-4 trials in RM, Phase 3 LYNX-1 trial in DLD and Phase 2b VEGA-1 trial for Nyxol as single agent and as adjunctive therapy with 0.4% low dose pilocarpine in presbyopia.

About Reversal of Mydriasis

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 and children. 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 treatment options to 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.

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.

About Ocuphire Pharma

Ocuphire is a publicly traded (Nasdaq: OCUP), clinical-stage, ophthalmic biopharmaceutical company focused on developing and commercializing small-molecule therapies for the treatment of refractive and retinal eye disorders.

Ocuphire has a previously disclosed partnership to develop and commercialize Nyxo[®] eye drops as a preservative-free eye drop formulation of phentolamine mesylate, a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size by uniquely blocking the alpha-1 receptors found only on the iris dilator muscle without affecting the ciliary muscle. Nyxol has been studied in a total of 12 clinical trials (3 Phase 1, 5 Phase 2, 4 Phase 3) across three indications, including single-use for reversal of pharmacologically-induced mydriasis (RM), and once-daily for treatment of presbyopia and dim light (night) vision disturbances (DLD), pending regulatory approvals. Nyxol's NDA under the 505(b)(2) pathway for the first indication RM has been accepted with a PDUFA date assigned of

September 28, 2023, and is currently in Phase 3 for presbyopia and DLD.

Ocuphire's other late-stage product candidate APX3330 is a first-in-class, small molecule, oral drug that blocks downstream pathways regulated by transcription factor Ref-1 – including those involving angiogenesis (VEGF) and inflammation (NFkB). These pathways are implicated across several ocular diseases, including diabetic retinopathy (DR), diabetic macular edema (DME), and age-related macular degeneration (AMD). Ocuphire recently announced topline data from the ZETA-1 Phase 2 trial in which APX3330 achieved statistical significance on a key pre-specified secondary endpoint of preventing clinically meaningful progression of (DR) after 24 weeks of daily treatment. APX3330 has also shown a favorable safety and tolerability profile in diabetic subjects (ZETA-1 trial) and in 11 previous clinical trials conducted in healthy, liver disease, and cancer subjects.

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 the potential receipt of regulatory approval for Nyxol for the treatment of RM, the potential to submit supplementary NDAs for presbyopia and DLD, the potential market opportunity for Nyxol, and Ocuphire's business strategy. 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) risks that the Nyxol partnership may not facilitate the commercialization or market acceptance of Ocuphire's product candidates; (x) the success and timing of commercialization of any of Ocuphire's product candidates and (xi) 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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