February 8, 2022

Ocuphire Completes Enrollment in MIRA-3 Pivotal Phase 3 Clinical Trial for Nyxol® in RM and Announces Two Upcoming Medical Conference Presentations

Completed enrollment ahead of schedule in second Phase 3 FDA registration trial for Nyxol in RM (MIRA-3) with top-line results expected soon around the end of 1Q22

VEGA-1 Phase 2 data showing the efficacy of Nyxol as a single-agent eye drop and together with low-dose pilocarpine for presbyopia to be presented at Cataract Surgery Meeting: Telling It Like It Is on February 10

Masked safety data from ongoing Phase 2 trial of APX3330 in DR to be presented at Angiogenesis, Exudation, and Degeneration 2022 Meeting on February 12

FARMINGTON HILLS, Mich., Feb. 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 MIRA-3, the second Phase 3 FDA registration trial evaluating the safety and efficacy of Nyxol eye drops to reverse pharmacologically-induced mydriasis (RM). In addition, APX3330 and Nyxol will have data presentations at each of the Angiogenesis, Exudation, and Degeneration 2022 Meeting and the Cataract Surgery Meeting: Telling It Like It Is.

“We are delighted to have surpassed our enrollment target of 330 with 368 patients in the MIRA-3 trial and accomplished this milestone ahead of schedule in less than 3 months,” stated Mina Sooch, MBA, CEO and Founder, Ocuphire Pharma. “We would like to thank our clinical investigators, their site staffs, and the study subjects for their participation in our clinical trial. This trial is part of the comprehensive MIRA clinical program to support a planned New Drug Application for Nyxol in RM later this year. There are no commercially available reversal drops for dilation, and Nyxol’s unique mechanism as an alpha-1 blocker has the potential to address an estimated $500 million RM market by providing therapeutic benefit to patients and utility for physicians in retinal, optometry and ophthalmology practices. Separately, we continue to share new safety and efficacy data from our ongoing Nyxol and APX3330 studies with the ophthalmic community and look forward to our presentations at the two upcoming medical conferences.”

Completed Enrollment in MIRA-3 Phase 3 Trial in Reversal of Mydriasis

Enrollment has been completed in just 11 weeks in the MIRA-3 Phase 3 clinical trial

investigating the safety and efficacy of Nyxol eye drops to reverse pharmacologically-induced mydriasis. The study, a randomized, double-masked, placebo-controlled pivotal Phase 3 study recruiting healthy subjects aged 12 and older, has exceeded its target enrollment of 330 subjects. A total of 368 patients were successfully enrolled in MIRA-3 across 16 centers in the US even with the challenging circumstances related to the COVID-19 pandemic. Oculos Development Services, a clinical research organization, led the execution of the MIRA-3 trial. Top-line results are expected to read out soon in early 2022 (around the end of this quarter). If successful, Ocuphire anticipates submission of a New Drug Application (NDA) with the U.S. Food and Drug Administration (FDA) for Nyxol for RM in late 2022.

Cataract Surgery: Telling It Like It Is Conference Presentation

Session:	New Technology Showdowns: Presbyopia-correcting Options
Title:	Nyxol (and Nyxol with LDP) for the Treatment of Presbyopia
Presenter:	Inder Paul Singh, MD
Date:	Thursday, February 10
Time:	2:30 – 3:00 PM EST
Location:	Orlando, FL

Dr. Singh will present for the first time to the medical community recently announced results from the completed VEGA-1 Phase 2 trial showing that Nyxol alone and Nyxol together with low-dose pilocarpine both had statistically significant improvements in near vision with a favorable safety profile that supports Phase 3 advancement as a single-agent drop and combination drops. Having both treatment options starting with Nyxol alone as the foundation would provide patients with the ability to “tune” pupil modulation to achieve near vision improvement based on individual and lifestyle needs.

Angiogenesis, Exudation, and Degeneration 2022 Conference Presentation

Session:	Session IX: Diabetic Retinopathy - Treatment
Title:	*APX3330, a Phase 2 Oral Drug Inhibiting Novel Target Ref-1 with a Dual Anti-VEGF and Anti-inflammatory MOA for the Treatment of Diabetic Retinal Diseases*
Presenter:	Peter K. Kaiser, MD
Date:	Saturday, February 12
Time:	12:00 – 12:10 PM EST
Location:	Virtual

Dr. Kaiser will present for the first time to the medical community safety data on oral APX3330 from the ongoing 24-week, multi-center, randomized, double-masked, placebo-controlled ZETA-1 Phase 2b trial in diabetic patients with retinal diseases. As announced during Ocuphire’s recent R&D Day, masked safety data from the study demonstrated a favorable safety profile consistent with prior studies for the first-in-class APX3330 oral drug, now with over 5,000 subject exposure days at 600 mg total daily dose across diabetic and cancer patients.

Reversal of Mydriasis Market Opportunity

Every year in the U.S., an estimated 100 million eyes are dilated 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. 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 options to safely reverse mydriasis. Nyxol has the potential to be the first and only FDA-approved agent for the reversal of Mydriasis.

Market research conducted by Global Data 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 investigational 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.

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 in March 2021 for MIRA-2, the first Phase 3 registration trial for treatment of RM, and recently initiated the second Phase 3 registration trial (MIRA-3) in RM. Ocuphire also reported positive top-line data in June 2021 for VEGA-1, a Phase 2 trial for the treatment of presbyopia. The company recently completed enrollment in its Phase 3 study of Nyxol for NVD and launched a pediatric trial in RM (MIRA-4). 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 ongoing second Phase 3 registration trial in RM (NCT05134974), MIRA-4 pediatric safety study in RM (NCT05223478) and 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) and enrollment in the Phase 3 registration trial in NVD (NCT04638660). 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.

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