

January 5, 2022



Ocuphire Provides Corporate Update: Announcing Enrollment Completion of Phase 3 Nyxol Trial, Enrollment Initiation of Nyxol Pediatric Trial, and an Investor R&D Day in January

Completed Enrollment of Nyxo[®] LYNX-1 Phase 3 NVD Trial

Initiated Enrollment of Nyxol MIRA-4 Pediatric Study in RM per Agreed Initial Pediatric Study Plan with FDA

Nyxol MIRA-3 Phase 3 Results, MIRA-4 Pediatric Results, and LYNX-1 Phase 3 Results Expected in Early 2022

Strengthened Balance Sheet Extends Runway into Q2 2023

Company to Host Virtual Investor R&D Day on January 31st

FARMINGTON HILLS, Mich., Jan. 05, 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 provided a corporate update available on the Company's website. This update includes recent progress on Nyxo[®] trials, the Company's cash position, and the announcement of an Investor R&D day in late January.

"We are looking forward to a catalyst-rich 2022 and the opportunity to build on the tremendous progress over the past year advancing our differentiated therapeutic candidates in front and back of the eye indications," stated Mina Sooch, MBA, President and CEO. "Our development program for Nyxol in the Reversal of Mydriasis (RM) indication is now in its final stages. We have recently agreed on an Initial Pediatric Study Plan (iPSP) with the FDA and began enrolling pediatric subjects ages 3 to 11 in the MIRA-4 study in late December. We also continue to enroll adults and 12 to 17 year-old subjects in MIRA-3, which is the second pivotal trial for the RM indication expected to read-out around the end of the first quarter. A positive outcome in MIRA-3 will position us to submit an NDA for Nyxol for RM in late 2022. We are also happy to report that this week marks the completion of over 140 subjects enrolled in LYNX-1, a Phase 3 pivotal trial for Nyxol in Night Vision Disturbances (NVD). We look forward to providing clinical updates on Nyxol in presbyopia and RM as well as APX3330 in diabetic retinopathy at our upcoming Virtual Investor R&D Day."

Initiated Enrollment in MIRA-4 Pediatric Trial in Reversal of Mydriasis: Ocuphire

recently enrolled the first subjects in MIRA-4, which is a randomized, double-masked, placebo-controlled study of Nyxol eye drops to reverse pharmacologically-induced mydriasis in healthy pediatric subjects. Approximately 20 pediatric subjects ages 3 to 11 will be enrolled with safety as the primary objective and efficacy as secondary objectives. Nyxol has the potential to address an estimated \$500 million reversal of dilation market across pediatrics and adults, which has no current commercially available therapies.

Completed Enrollment of LYNX-1 Study in Night Vision Disturbances: Enrollment has been completed in the LYNX-1 Phase 3 clinical trial investigating Nyxol for the treatment of NVD. LYNX-1 is a randomized, double-masked, placebo-controlled registration study designed to evaluate the safety and efficacy of Nyxol compared to placebo in patients with NVD. NVD, also known as dim light vision disturbances (DLD), is a condition in which peripheral imperfections (aberrations) of the cornea scatter light when the pupil naturally dilates in dim light conditions. Patients with NVD commonly experience visually impeding glare, halos, starbursts and decreased contrast sensitivity. Based on GlobalData market research, about 38 million individuals in the US are believed to suffer from NVD. An estimated 16 million individuals have moderate-to-severe NVD that may benefit from Nyxol's ability to reduce the pupil diameter and provide better night vision by eliminating the peripheral aberrations.

Key Anticipated 2022 Milestones:

- **Reversal of Mydriasis (RM):** Report top-line results in early 2022 from the Nyxol Phase 3 MIRA-3 registration trial and the MIRA-4 pediatric trial; Planning to file an NDA with FDA for Nyxol in RM indication in late 2022
- **Presbyopia:** Initiate Phase 3 program (VEGA 2/VEGA 3) in 1H 2022 investigating Nyxol and low-dose pilocarpine (LDP)
- **Night Vision Disturbances (NVD):** Report top-line results in early 2022 from the Nyxol Phase 3 LYNX-1 trial
- **Diabetic Retinopathy (DR) and Diabetic Macular Edema (DME):** Report top-line results in 2H 2022 from the APX3330 Phase 2 ZETA-1 trial

\$24.5M Cash at Year End: As of December 31, 2021, Ocuphire had cash and cash equivalents of approximately \$24.5 million. We expect that our strengthened balance sheet will support operations into the second quarter of 2023, as compared to previous guidance of late 2022. Ocuphire had 18.8 million shares of common stock outstanding as of year-end.

Panel Discussion at LifeSci Partners Corporate Access Event on January 6, 2022:

Mina Souch, MBA, President, CEO and Founder, will participate in a virtual panel discussion "The Role of Gender Equality in Changing the Landscape of Life Sciences Innovation & Investment" during the LifeSci Partners 11th Annual Corporate Access Event on Thursday, January 6th, 12:00 to 12:55pm ET. To access the panel, please register [here](#).

Company to Host Investor R&D Day on Monday, January 31, 2022: Ocuphire will host a Virtual Investor R&D Day for the investment community at which six ophthalmic Key Opinion Leaders (KOLs) from retina, optometry and refractive surgery practices will share their thoughts on three large unmet indications, RM, presbyopia, and DR/DME, addressed by

Ocuphire's two late-stage clinical drug assets and provide status updates on the development programs for Nyxol and APX3330. The event will take place from 10:00am to 12:00pm ET on Monday, January 31st and will feature insights from David Boyer, M.D., Peter Kaiser, M.D., Paul M. Karpecki, O.D., F.A.A.O., James Katz, M.D., Mitchell Jackson, M.D., and Jay S. Pepose, M.D., Ph.D. To access the event, please register [here](#).

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 refractive and retinal eye disorders. Ocuphire's pipeline currently includes two small-molecule product candidates targeting multiple front and back of the eye indications. The company's lead product candidate, Nyxol® (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 clinical trials. 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 well-controlled Phase 2 trial for the treatment of presbyopia. The Phase 3 clinical trial for Nyxol in NVD patients (LYNX-1) also recently fully enrolled. 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. APX3330 is currently enrolling subjects in a Phase 2 clinical trial in subjects with 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 ongoing 2nd Phase 3 registration trial in RM ([NCT05134974](https://clinicaltrials.gov/ct2/show/study/NCT05134974)) and Phase 2 trial in DR/DME ([NCT04692688](https://clinicaltrials.gov/ct2/show/study/NCT04692688)). For more information on the recently completed trials, see the links to the 1st Phase 3 registration trial in RM ([NCT04620213](https://clinicaltrials.gov/ct2/show/study/NCT04620213)), Phase 2 trial in presbyopia ([NCT04675151](https://clinicaltrials.gov/ct2/show/study/NCT04675151)), and Phase 3 registration trial in NVD ([NCT04638660](https://clinicaltrials.gov/ct2/show/study/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 the future clinical trials in RM, presbyopia, NVD and DR/DME, and statements regarding cash runway. 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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