

January 31, 2022



Ocuphire Presenting New Data and Updates on APX3330 and Nyxol® Clinical Programs at Virtual Investor R&D Day

New data support Phase 3 development of Nyxol alone as a durable single eye drop for the potential treatment of Presbyopia

First update on recruitment in APX3330 ZETA-1 Phase 2b trial: ~70% patients enrolled

New masked data show favorable safety profile for oral APX3330 in diabetic patients

Advancing pre-commercial activities for Nyxol in RM with expected NDA filing late 2022 and potential market commercialization in 2023

Live R&D Day webinar featuring six leading ophthalmic KOLs today @ 10am ET

FARMINGTON HILLS, Mich., Jan. 31, 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, is hosting a virtual investor R&D Day for the investment community today, Monday, January 31 from 10:00am to 12:15pm ET with live Q&A following each session.

The Investor R&D Day will feature six ophthalmic Key Opinion Leaders (KOLs) from refractive surgery, optometry and retina practice areas who will share their thoughts on three large unmet indications addressed by Ocuphire's two late-stage clinical drug assets.

Ocuphire's lead product candidate, Nyxol eye drops (0.75% phentolamine ophthalmic solution), is a non-selective alpha-1 and alpha-2 adrenergic inhibitor for the treatment of refractive conditions; its second product candidate, APX3330, is a first-in-class oral Ref-1 inhibitor with a novel dual anti-VEGF and anti-inflammatory mechanism for the treatment of diabetic retinal diseases.

To access the R&D Day presentations live or for the recording, please click [here](#).

Investor R&D Day Agenda

Session I: APX3330 in Diabetic Retinopathy (DR)

Led by KOLs Mark Kelley, Ph.D., Peter Kaiser, M.D., and David Boyer, M.D.

- First update on enrollment in the randomized, double-masked, placebo-controlled, multi-center, 24-week Phase 2b ZETA-1 trial with approximately 70% of target DR/DME patients enrolled, and on track for expected data in 2H 2022

- New masked safety data from the ZETA-1 study demonstrated a favorable safety profile consistent with prior studies for the first-in-class APX3330 oral drug with additional exposure data in diabetic patients with retinal disease

Session II: Nyxol for Reversal of Mydriasis (RM)

Led by KOLs Paul Karpecki, O.D., F.A.A.O. and Mitchell Jackson, M.D.

- Nyxol met its primary endpoint in its first Phase 3 RM registration trial demonstrating statistically significant rapid reversal of dilation with an alpha-1 adrenergic blocker MOA uniquely suited to avoid the narrow-angle and other safety issues associated with cholinergic drug (e.g., pilocarpine) reversal of dilations
- With an opportunity to impact over 100 million eye dilations annually for routine eye exams, surgical procedures, and retina treatments, pre-commercial activities are underway to support the potential launch of Nyxol in RM in 2023 led by Bindu Manne, Ocuphire's recently appointed Head of Market Development and Commercialization

Session III: Nyxol in Presbyopia

Led by KOLs Jay Pepose, M.D., Ph.D. and James Katz, M.D.

- New data from the Phase 2 VEGA-1 clinical trial show that Nyxol had statistically significant improvement in efficacy and long durability compared to placebo at 12 hours post-dosing supporting future clinical development as a single drop
- Given both treatment options would provide patients with flexibility to achieve near vision improvement based on their lifestyle needs, Ocuphire plans to proceed with the Phase 3 VEGA program to file New Drug Applications (NDAs) for both Nyxol as a single drop and with low-dose pilocarpine (LDP) as adjunctive treatment, and, assuming successful trials, expects to file in 2023

"We are excited to host the Investor R&D Day today with our prestigious panel of KOLs and share new data from the ongoing APX3330 Phase 2 trial and the completed Nyxol presbyopia Phase 2 trial as well as physician insights on the large unmet needs in our target refractive and retinal indications for Nyxol and APX3330," stated Mina Sooch, MBA, CEO and Founder, Ocuphire Pharma. "We are looking forward to continuing our tremendous progress with these small molecule therapeutic drug candidates with an even more catalyst-rich 2022 as we advance these therapies to patients."

Key Anticipated Milestones for 2022:

- **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; Plans to file an NDA with FDA for Nyxol in RM indication in late 2022
- **Presbyopia:** Reporting statistically significant efficacy and durability with Nyxol alone in January 2022; Plans to initiate VEGA Phase 3 program in mid-2022 investigating Nyxol alone and Nyxol with low-dose pilocarpine (LDP) as adjunctive treatment
- **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 from the APX3330 Phase 2b ZETA-1 trial in 2H 2022

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](https://clinicaltrials.gov/ct2/show/study/NCT05134974)), and Phase 2b trial in DR/DME ([NCT04692688](https://clinicaltrials.gov/ct2/show/study/NCT04692688)). Ocuphire previously completed the first 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 enrollment in the 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 regulatory timelines, commercial timelines, 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.

Ocuphire Contacts

Mina Sooch, President & CEO

Ocuphire Pharma, Inc.

ir@ocuphire.com

www.ocuphire.com

Corey Davis, Ph.D.

LifeSci Advisors

cdavis@lifesciadvisors.com



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