

November 1, 2021



Ocuphire Invited to Present Clinical Data on Nyxol® and APX3330 at the American Academy of Ophthalmology 2021 Annual Meeting and Eyecelerator/AAO Meeting

FDA Approves First Pharmaceutical Therapy to Treat Presbyopia; Nyxol on Track to Start Phase 3 Presbyopia Trials in First Half of 2022

Ocuphire Uniquely Positioned in Developing Late-Stage Innovative Therapies for Retinal Disease and Presbyopia

FARMINGTON HILLS, Mich., Nov. 01, 2021 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of several eye disorders, today announced that clinical data on its therapeutic candidates Nyxol® and APX3330 will be featured in poster sessions as presentations at the upcoming [American Academy of Ophthalmology \(AAO\) 2021 annual meeting](#) to take place in New Orleans, November 12 – 15. In addition, Ocuphire will present at the [Eyecelerator@AAO 2021](#) satellite symposium on November 11.

“We are delighted to see the early US regulatory approval of Allergan’s eye drops, the first pharmaceutical therapy to be approved for the treatment of presbyopia,” said Mina Sooch, President & CEO of Ocuphire. “Presbyopia represents an area of significant growth and market opportunity with over 120 million Americans living with presbyopia and dependent on reading glasses. Based upon our positive Phase 2 study results reported this year, we believe that Nyxol and low dose pilocarpine (LDP) represents a novel mechanism of action with a differentiated safety, efficacy, and durability profile. We plan to initiate our Phase 3 trials for presbyopia in the first half of 2022. We believe that Nyxol’s clinical profile holds the potential as a best-in-class therapy for the treatment of presbyopia.”

AAO 2021 Subspecialty Day Presentation Details

Title: **Favorable Safety and Tolerability Profile of Oral APX3330 Drives Dosing Strategy for Ongoing Phase 2 Trial for DR/DME**

Subspecialty Day: Retina, Friday and Saturday, November 12-13, 2021

Abstract#: PO332 ([link](#))

Presenting author: Michael J. Allingham, MD, PhD

Dr. Allingham will present safety data on oral APX3330 from over 300 healthy volunteers and patients with chronic hepatitis across five Phase 1 and five Phase 2 clinical trials at

doses up to 600 mg/day. A sixth Phase 1 study will also be presented, showing safety data from 19 patients with solid tumors who were treated with daily oral dosing of APX3330 of up to 720 mg/day. Additional preclinical data on MOA in normal cells and PK exposure data will be presented. The aggregate clinical data from these 11 completed trials support Ocuphire's ZETA-1 Phase 2b study, an ongoing, randomized, double-masked, placebo-controlled trial evaluating the safety and efficacy of oral APX3330 in the treatment of diabetic retinopathy (DR) and diabetic macular edema (DME). Additional information about the ZETA-1 Phase 2b trial can be found at [www.clinicaltrials.gov \(NCT04692688\)](https://www.clinicaltrials.gov/ct2/show/study/NCT04692688).

Title: Phase 2 Clinical Trial to Evaluate the Efficacy of Phentolamine Ophthalmic Solution and Low-Dose Pilocarpine for the Treatment of Presbyopia

Subspecialty Day: Refractive Surgery, Friday and Saturday, November 12-13, 2021

Abstract #: 30068457 ([link](#))

Presenting Author: Jay S. Pepose, MD, PhD

Dr. Pepose will present results from the VEGA-1 Phase 2 multi-center, randomized, placebo-controlled, double-masked clinical trial which evaluated a kit combination of Nyxol, 0.75% phentolamine ophthalmic solution (POS), plus low-dose 0.4% pilocarpine (LDP) for the treatment of presbyopia. Ocuphire reported top line positive data from VEGA-1 earlier this year. The VEGA-1 study enrolled 150 presbyopic patients at 17 investigational sites in the US. On the primary endpoint, 61% of subjects treated with Nyxol + LDP improved 15 letters or greater (≥ 3 lines) in photopic binocular near vision at 1 hour compared with 28% of subjects on placebo (33% placebo adjusted difference; $p=0.003$). Treatment with Nyxol and/or LDP did not reduce best corrected distance vision. Moreover, Nyxol and/or LDP was well tolerated with no headaches and had a durable effect measured up to 6 hours. Additional information about the VEGA-1 Phase 2 trial can be found at [www.clinicaltrials.gov \(NCT04675151\)](https://www.clinicaltrials.gov/ct2/show/study/NCT04675151).

Eyecelerator@AAO 2021

Company: Retina, Cornea, Glaucoma & Anterior Segment

Showcase:

Session Room: Glaucoma & Anterior Segment

Time: 11:30am Central US / 12:30pm Eastern US

Date: November 11, 2021

Presenter: Mina Sooch, M.B.A., CEO Ocuphire

Eyecelerator is a partnership between the American Academy of Ophthalmology and American Society of Cataract and Refractive Surgery (ASCRS) to accelerate ophthalmic innovation through next-generation business conferences and platforms. Ocuphire is part of a lineup of more than 30 companies, from startups to public, who will present innovations at all stages. The format will be five-minute company presentations followed by a moderated Q+A with panels of seasoned pharma executive, investors, and KOLs. Ocuphire will be one of two companies presenting clinical data for Presbyopia at the meeting. For more details, please visit <https://www.eyecelerator.com>

About Ocuphire Pharma

Ocuphire is a publicly traded (NASDAQ: OCUP), clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for refractive and retinal eye disorders. Ocuphire's pipeline currently includes two small-molecule product candidates targeting front and back of the eye 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 dim light or night vision disturbances (NVD), reversal of pharmacologically-induced mydriasis (RM), and presbyopia, and has been studied in 9 clinical trials including the recently completed Phase 3 trial in RM and Phase 2 trial in presbyopia. Ocuphire reported positive top-line data in March 2021 for MIRA-2, a Phase 3 FDA registration study for treatment of RM. Ocuphire also reported positive top-line data in June 2021 for VEGA-1, a Phase 2 trial for the treatment of presbyopia. Nyxol is also currently in Phase 3 clinical development 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. 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 completed Phase 2 trials, recently completed Phase 3 registration trial in RM ([NCT04620213](https://clinicaltrials.gov/ct2/show/study/NCT04620213)), recently completed Phase 2 trial in presbyopia ([NCT04675151](https://clinicaltrials.gov/ct2/show/study/NCT04675151)), ongoing Phase 3 registration trial in NVD ([NCT04638660](https://clinicaltrials.gov/ct2/show/study/NCT04638660)), and Phase 2 trial in DR/DME ([NCT04692688](https://clinicaltrials.gov/ct2/show/study/NCT04692688)). For more information, please 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 Nyxol plus LDP's potential to be a 'best-in-class' presbyopia treatment option, the US and global market and commercial potential of Nyxol alone or in combination with LDP, and the expected timing of our future clinical trials in RM, NVD, presbyopia, 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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Source: Ocuphire Pharma