February 16, 2023

Ocuphire Announces APX3330 Phase 2 Data Presentations at Retina Meetings

Topline Data from ZETA-1 Phase 2 Trial of Oral APX3330 in Diabetic Retinopathy Presented on February 11th at 20th Angiogenesis, Exudation, and Degeneration 2023 Meeting

Topline Data from ZETA-1 Accepted for Presentation on February 17th at Macula Society 46th Annual Meeting

Presentations Highlight Binocular 3-Step or More DRSS Worsening Measure in the ZETA-1 Phase 2 Trial; This Pre-Specified Secondary Endpoint Met by APX3330 is the Planned Potential Phase 3 Primary Registration Endpoint

FARMINGTON HILLS, Mich., Feb. 16, 2023 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of refractive and retinal eye disorders, today announced two presentations featuring efficacy and safety results from the Company's recently completed ZETA-1 Phase 2 trial of oral APX3330 in diabetic retinopathy (DR) at two medical meetings in February. Peter Kaiser, M.D., Professor of Ophthalmology at the Cleveland Clinic Lerner College of Medicine and Cole Eye Institute, presented the results at the Angiogenesis, Exudation, and Degeneration 2023 Meeting held virtually on February 10-11, 2023. Rishi P. Singh, M.D., President of Cleveland Clinic Martin North and South hospitals, and Professor of Ophthalmology at the Lerner College of Medicine, will present at the upcoming Macula Society 46th Annual Meeting, to be held in-person February 15-18, 2023 in Miami, FL.

Dr. Kaiser's presentation, titled "Efficacy and Safety Data for APX3330, a Novel Oral Drug Candidate for DR/DME, from the ZETA-1 Phase 2 Trial," discussed results from the trial during the Diabetic Retinopathy Imaging and Treatment session at the 20th Angiogenesis, Exudation, and Degeneration 2023 Meeting on Saturday, February 11th organized by the Bascom Palmer Eye Institute. Oral APX3330 achieved statistical significance on a key pre-specified secondary DRSS (diabetic retinopathy severity scale) endpoint of preventing clinically meaningful progression of DR after 24 weeks of treatment. In addition, APX3330 demonstrated a favorable systemic and ocular safety and tolerability profile.

Dr. Kaiser noted, "The Phase 2 clinical trial results of the oral drug candidate APX3330 that were presented at the Angiogenesis 2023 meeting showed favorable efficacy, safety, and tolerability. Intravitreal injections are currently approved for DR based on 2-step improvement in DRSS in one eye. What was new for many retina specialists is that for a systemic medication that works in both eyes, the use of binocular improvement or prevention of worsening in DRSS is an acceptable endpoint for FDA approval. We would prefer to treat our DR patients early and non-invasively, so an oral treatment option that prevents

worsening of DR would be very appealing."

Presentation details for the Macula Society 46th Annual Meeting presentation can be found below:

Title:	**Safety and Efficacy of an Oral Therapeutic APX3330 from ZETA-1 Phase 2 Trial in Patients with Diabetic Retinopathy**
Session:	Diabetic Retinopathy II
Date/Time:	Friday, February 17, 2023, 9:41 AM ET
Presenting Author:	Rishi P. Singh, M.D.
Location:	Fontainebleau Miami, FL
Link:	Macula Society 46th Annual Meeting

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 Nyxol® 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. 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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Source: Ocuphire Pharma