May 6, 2024

Ocuphire Pharma Announces Presentation on APX3330 at the ARVO 2024 Annual Meeting

FARMINGTON HILLS, Mich., May 06, 2024 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP) (“Ocuphire”), a clinical-stage biopharmaceutical company focused on developing novel therapies for the treatment of retinal and refractive eye disorders, today announced that clinical data from its ZETA-1 trial evaluating APX3330 in diabetic retinopathy (DR) on a validated binocular person-level scale was presented yesterday at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting, taking place May 5-9, 2024 in Seattle, Washington. The presentation, titled *Oral APX3330, a Ref-1 Inhibitor, Slows Progression of Diabetic Retinopathy on a Binocular DRSS Person-Level Scale*, was delivered by Daniel Su, M.D., a practicing retina specialist at Retina-Vitreous Associates Medical Group (LA Retina) in Los Angeles, California.

Presentation Highlights:

- ZETA-1 was a Phase 2, randomized, double-masked trial evaluating the efficacy and safety of oral APX3330 compared to placebo in 103 participants with DR completed in January 2023.
- A subset analysis was conducted to evaluate the efficacy of APX3330 in slowing DR progression using the target population of the planned Phase 2/3 study and the Food and Drug Administration’s (the FDA)-agreed upon registration endpoint of a 3-step change on a binocular diabetic Retinopathy Severity Scale (DRSS). This 17-step person-level scale accounts for the DRSS scores of the two eyes and then anchors the step to the worse eye. The subset comprised 68 participants from the ZETA-1 trial who had a baseline DRSS score of 47 or 53 in at least one eye and 43, 47 or 53 in the other eye.
- Analysis of the ZETA-1 Phase 2 subset using the binocular person-level scale showed that no participants in the APX3330 group had a ≥ 4-step worsening at week 24 compared to 15.2% in the placebo group, representing a 100% reduction between groups (p=0.07). Similarly, only 5.7% of APX3330-treated subjects had a ≥ 3-step worsening at week 24 compared to 15.2% of placebo subjects, representing a 62.5% reduction between groups (p=0.26).
- Fewer participants in the APX3330 group developed proliferative diabetic retinopathy (PDR) by week 24 compared to the placebo group (11% vs 26% respectively; p=0.13).
- APX3330 showed favorable safety and tolerability, with similar ocular adverse events between APX3330 and placebo groups.

“We were pleased to present at ARVO results from the ZETA-1 Phase 2 trial analyzed using both the target population and the FDA-agreed upon registration endpoint for the planned ZETA-2 Phase 2/3 trial,” said George Magrath, M.D., M.B.A., M.S., Chief Executive Officer of Ocuphire. “Results from this subset of participants highlight that APX3330 meaningfully slows DR worsening in patients with moderate to very severe non-proliferative DR (NPDR) in both eyes, representing those who are at higher risk for developing proliferative DR. We are working closely with the FDA regarding our Special Protocol Assessment and aim to finalize the Phase 2/3 protocol soon. We believe that APX3330 has significant potential as a promising oral binocular treatment option for delaying or preventing DR progression in patients who currently lack effective options prior to developing vision-threatening complications.”

About Ocuphire Pharma

Ocuphire is a clinical-stage ophthalmic biopharmaceutical company focused on developing novel therapies for the treatment of retinal and refractive eye disorders.

Ocuphire’s lead retinal product candidate, APX3330, is an oral small-molecule inhibitor of Ref-1 (reduction oxidation effector factor-1 protein) for the treatment of non-proliferative diabetic retinopathy (NPDR). Ref-1 is a regulator of the transcription factors HIF-1α and NF-κB. Inhibiting REF-1 reduces levels of vascular endothelial growth factor (VEGF) and inflammatory cytokines which are known to play key roles in ocular angiogenesis and inflammation. APX3330 is an oral tablet to be administered twice per day for the treatment of diabetic retinopathy (DR). A Phase 2 study in subjects with DR and an End-of-Phase 2 meeting have been completed, and a special protocol assessment (SPA) was submitted to the U.S. Food and Drug Administration (FDA) in February 2024.

In addition, Ocuphire has a partnership with Viatris, Inc. (“Viatris”) to develop and commercialize Phentolamine Ophthalmic Solution 0.75% (“PS”), a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size. PS was approved by the FDA for the treatment for pharmacologically-induced mydriasis under the brand name RYZUMVI™ in September 2023. PS is also in Phase 3 clinical development for the treatment of presbyopia and for the treatment of decreased visual acuity under low light (mesopic) conditions after keratorefractive surgery.

Ocuphire is also developing APX2009 and APX2014, second-generation analogs of APX3330. These programs are being evaluated for treating other retinal diseases such as age-related macular degeneration and geographic atrophy. For more information, please visit www.ocuphire.com.

Forward Looking Statements

This press release contains 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 efficacy of APX3330 in slowing the progression of diabetic retinopathy, the safety and tolerability of APX3330, ongoing discussions with the FDA regarding various of our drug products, and continued drug development under our agreement with Viatris.

These forward-looking statements relate to us, our business prospects and our results of

operations and are subject to certain risks and uncertainties posed by many factors and events that could cause our actual business, prospects and results of operations to differ materially from those anticipated by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those described under the heading “Risk Factors” included in our Annual Report on Form 10-K. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. In some cases, you can identify forward-looking statements by the following words: “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “aim”, “may,” “ongoing,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. We undertake no obligation to revise any forward-looking statements in order to reflect events or circumstances that might subsequently arise.

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:

- The success and timing of regulatory submissions and pre-clinical and clinical trials, including enrollment and data readouts;
- Regulatory requirements or developments;
- Changes to or unanticipated events in connection with clinical trial designs and regulatory pathways;
- Delays or difficulties in the enrollment of patients in clinical trials;
- Substantial competition and rapid technological change;
- Our development of sales and marketing infrastructure;
- Future revenue losses and profitability;
- Our relatively short operating history;
- Changes in capital resource requirements;
- Risks related to the inability of Ocuphire to obtain sufficient additional capital to continue to advance its product candidates and its preclinical programs;
- Domestic and worldwide legislative, regulatory, political and economic developments;
- Employee misconduct;
- Changes in market opportunities and acceptance;
- Reliance on third-parties;
- Future, potential product liability and securities litigation;
- System failures, unplanned events, or cyber incidents;
- The substantial number of shares subject to potential issuance associated with our equity line of credit arrangement;
- Risks that our partnership with Viatris, or our other licensing arrangements, may not facilitate the commercialization or market acceptance of Ocuphire’s product candidates;
- Future fluctuations in the market price of our common stock;
- The success and timing of commercialization of any of Ocuphire’s product candidates; and
- Obtaining and maintaining 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. Readers are urged to carefully review and consider the various disclosures made by us in this report and in our other reports filed with the Securities and Exchange Commission that advise interested parties of the risks and factors that may affect our business. 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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