August 11, 2023

Ocuphire Pharma Announces Financial Results for Second Quarter 2023 and Provides Corporate Update

End-of-Phase 2 Meeting with FDA Scheduled for Q4 2023 for Oral APX3330 in Diabetic Retinopathy (DR)

Cash Balance of $40 Million Expected to Fund Operations into 2025

Nyxol RM PDUFA date September 28, 2023; Approval would trigger $10 million milestone payment to Ocuphire

Common Share Purchase Agreement with Lincoln Park Capital Fund, LLC providing up to $50 million in committed financing over 30 months

FARMINGTON HILLS, Mich., Aug. 11, 2023 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing small-molecule therapies for the treatment of retinal and refractive eye disorders, today announced financial results for the second quarter ended June 30, 2023, and provided a corporate update.

“We continue to advance the development of oral APX3330 for diabetic retinopathy, as we prepare for our End-of-Phase 2 meeting with the FDA in the fourth quarter of 2023,” said Rick Rodgers, Interim Chief Executive Officer. “We intend to confirm with the Agency our Phase 3 registration endpoint and path to a potential approval. In the meantime, we continue to present at prominent medical forums the results from the ZETA-1 Phase 2 trial of APX3330, which demonstrated prevention of 3-step binocular worsening as measured on the diabetic retinopathy severity scale, our anticipated Phase 3 registration endpoint. If approved, we believe that APX3330 has the potential to shift the current wait-and-watch deferred treatment paradigm for NPDR without DME and be the first non-invasive, oral, early treatment for the 8 million NPDR patients who are at risk of progressing to vision-threatening complications. We are also pleased to enter into a common share purchase agreement with Lincoln Park Capital Fund, providing us with additional financial flexibility to fund further development of APX3330.”

Key Anticipated Future Milestones

- **APX3330:** End-of-Phase 2 (EOP2) meeting scheduled with FDA to confirm Phase 3 regulatory path for oral APX3330 in DR (Q4 2023)
- **Nyxol:** PDUFA date for Nyxol in RM (September 28, 2023). FDA approval in RM would trigger a $10 million milestone payment to Ocuphire
- **Nyxol:** Topline results from VEGA-2 Phase 3 pivotal trial of Nyxol in Presbyopia (Q4

2023)

Recent Business Highlights

Clinical and Regulatory Updates

In July 2023, Ocuphire confirmed an End-of-Phase 2 meeting with the FDA, scheduled for the fourth quarter of 2023. On the strength of the efficacy and safety data from the ZETA-1 Phase 2 trial of APX3330, and upon agreement with the FDA following the End-of-Phase 2 meeting, Ocuphire plans to pursue binocular 3-step or more worsening (prevention of progression) of diabetic retinopathy severity score (DRSS) as the potential registration primary endpoint.

Corporate

On August 10, 2023, Ocuphire entered into a common share purchase agreement (the "Purchase Agreement") with Lincoln Park Capital Fund, LLC (LPC), an institutional investor. Subject to the terms and conditions of the Purchase Agreement, Ocuphire has the right to sell, and LPC is obligated to purchase, up to $50 million of Ocuphire's common shares over a 30-month period at prices per share as computed under the Purchase Agreement. Ocuphire, in its sole discretion, controls the timing and amount of all sales of common shares within a pre-specified range and there are no warrants or other share classes associated with the Purchase Agreement. Proceeds from share sales are expected to fund future development of APX3330 and to be used for general corporate purposes.

Additional information regarding the Purchase Agreement with LPC will be available in a Current Report on Form 8-K to be filed with the Securities and Exchange Commission.

Presentations, Publications, and Conferences

In the second quarter of 2023, several papers, posters, and panel talks were presented at medical and industry conferences with updates on APX3330 in DR and Nyxol in RM, DLD and Presbyopia. Highlights include:

- A late-breaking poster and ePoster theater presentation featured results from ZETA-1 Phase 2 trial of APX3330 in DR at the 83rd Scientific Sessions of the American Diabetes Association (ADA) in June 2023.
- Previously announced results from ZETA-1 Phase 2 trial of APX3330 in DR were presented to the medical community at the ASCRS Annual Meeting in May 2023.
- Results from LYNX-1 Phase 3 trial of Nyxol in DLD were presented for the first time to the medical community at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in April 2023, and at the American Society of Cataract and Refractive Surgery (ASCRS) Annual Meeting in May 2023.

Second Quarter ended June 30, 2023 Financial Highlights

As of June 30, 2023, Ocuphire had cash and cash equivalents of approximately $40.0 million. The Company has no debt. Based on current projections, management believes the present cash on hand will be sufficient to fund operations into 2025.

License and collaborations revenue was $3.7 million and $5.4 million for the three and six

months ended June 30, 2023, respectively. There was no license and collaborations revenue during the three and six months ended June 30, 2022. Revenue during the current year three and six month periods was derived primarily from the reimbursement of research and development services under the Nyxol License Agreement.

General and administrative expenses for the three and six months ended June 30, 2023 were $4.3 million and $6.6 million, respectively, compared to $1.8 million and $3.5 million, respectively, for the three and six months ended June 30, 2022. The increases from the comparable periods in 2022 were attributable to severance costs associated with the departure of our former Chief Executive Officer, stock-based compensation, professional services, legal support and personnel related and other costs. General and administrative expenses included stock-based compensation expenses.

Research and development expenses for the three and six months ended June 30, 2023 were $4.7 million and $10.3 million, respectively, compared to $3.2 million and $7.9 million, respectively, for the three and six months ended June 30, 2022. The increases from the comparable periods in 2022 were primarily attributable to increased clinical costs of for Nyxol and APX3330 period over period as well as increased consulting and other costs during the current period. Research and development expenses also included stock-based compensation expenses.

The loss from operations for the three and six months ended June 30, 2023, was $5.4 million and $11.5 million, respectively, compared $4.9 million and $11.4 million, respectively, for the three and six months ended June 30, 2022.

Net loss for the three and six months ended June 30, 2023, was $5.0 million and $10.8 million, respectively, compared to $4.9 million and $11.5 million, respectively, for the three and six months ended June 30, 2022. Net loss per share for the three and six months ended June 30, 2023, was ($0.24) and ($0.51) per share, respectively, compared to ($0.25) and ($0.60) per share, respectively, for the comparable periods in 2022.

For further details on Ocuphire's financial results, refer to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2023 to be filed with the Securities and Exchange Commission.

About Ocuphire Pharma

Ocuphire Pharma, Inc. is a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing small-molecule therapies for the treatment of retinal and refractive eye disorders.

Ocuphire's lead retinal product candidate, APX3330, is a first-in-class small-molecule inhibitor of Ref-1 (reduction oxidation effector factor-1 protein). Ref-1 is a regulator of transcription factors such as HIF-1a and NF-kB. 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. Through inhibition of Ref-1, APX3330 normalizes the levels of VEGF to physiologic levels, unlike biologics that abolish the VEGF levels required for normal function. APX3330 is an oral tablet administered twice per day for the treatment of diabetic retinopathy ("DR") and diabetic macular edema ("DME"). A Phase 2 study in subjects with DR or DME has recently completed, and an End-of-Phase 2 meeting is

confirmed with the FDA in Q4 2023.

DR affects approximately 10 million people with diabetes and is projected to impact 14.6 million Americans by 2050. DR is classified as Non-Proliferative Diabetic Retinopathy ("NPDR"), the early stage of the disease in which symptoms may be mild or nonexistent or Proliferative Diabetic Retinopathy ("PDR") which is the more advanced stage of diabetic eye disease that can be highly symptomatic with loss of vision. Approximately 80% of DR patients have NPDR that will progress to PDR if left untreated. Despite the risk for visual loss associated with this disease, over 90% of NPDR patients currently receive no course of treatment apart from observation by their eye care specialist until they develop sight-threatening complications. This is due to the burdensome and frequent eye injections currently required with currently approved therapies for this disease. APX3330 as an oral tablet has the potential to be an early, non-invasive treatment for the 8 million NPDR patients in the US. Treatment with APX3330 is expected to delay or prevent progression of NPDR, thereby reducing the need for expensive intravitreal injections with anti-VEGF therapies and reducing the likelihood of vision loss due to DR.

Ocuphire has also in-licensed APX2009 and APX2014, which are second-generation analogs of APX3330. The unique dual mechanism of action of these Ref-1 inhibitors of reducing angiogenesis and inflammation could potentially be beneficial in treating other retinal diseases such as age-related macular degeneration ("AMD"), and geographic atrophy ("GA"). Ocuphire is currently evaluating local delivery routes in addition to the systemic (oral) route as part of its pipeline expansion in retinal therapies.

Ocuphire has a partnership with Viatris, Inc. 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 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. Nyxol is currently in Phase 3 for presbyopia and DLD.

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 End-of-Phase 2 meeting with the FDA to confirm Phase 3 registration endpoints and study parameters, and 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.

Contacts

Corporate	Investor Relations
Rick Rodgers Interim President & CEO ir@ocuphire.com	Corey Davis, Ph.D. LifeSci Advisors cdavis@lifesciadvisors.com

Ocuphire Pharma, Inc.
Condensed Balance Sheets
(in thousands, except share amounts and par value)

	As of	
	June 30, 2023 (unaudited)	**December 31, 2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 39,977	$ 42,634
Accounts receivable	135	1,298
	2,595	3,552
Contract assets and unbilled receivables		
Prepaids and other current assets	732	1,453
Short-term investments	22	49
Total current assets	43,461	48,986
Property and equipment, net	4	6
Total assets	$ 43,465	$ 48,992

Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,323	$ 1,069
Accrued expenses	3,438	1,684
Total current liabilities	5,761	2,753
Total liabilities	5,761	2,753
Commitments and contingencies		
Stockholders' equity		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of June 30, 2023 and December 31, 2022; no shares issued and outstanding at June 30, 2023 and December 31, 2022.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of June 30, 2023 and December 31, 2022; 20,985,784 and 20,861,315 shares issued and outstanding at June 30, 2023 and December 31, 2022, respectively.	2	2
Additional paid-in capital	119,934	117,717
Accumulated deficit	(82,232)	(71,480)
Total stockholders' equity	37,704	46,239
Total liabilities and stockholders' equity	$ 43,465	$ 48,992

Ocuphire Pharma, Inc.
Condensed Statements of Comprehensive Loss
(in thousands, except share and per share amounts)
(Unaudited)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	**2022**	**2023**	**2022**
License and collaborations revenue	$ 3,674	$ —	$ 5,423	$ —
Operating expenses:				
General and administrative	4,340	1,776	6,625	3,512
Research and development	4,723	3,162	10,318	7,934
Total operating expenses	9,063	4,938	16,943	11,446
Loss from operations	(5,389)	(4,938)	(11,520)	(11,446)

Interest expense	—	(4)	—	(9)
Other income (expense), net	428	15	768	(67)
Loss before income taxes	(4,961)	(4,927)	(10,752)	(11,522)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(4,961)	(4,927)	(10,752)	(11,522)
Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (4,961)	$ (4,927)	$ (10,752)	$ (11,522)
Net loss per share:				
Basic and diluted	$ (0.24)	$ (0.25)	$ (0.51)	$ (0.60)
Number of shares used in per share calculations:				
Basic and diluted	20,959,807	19,502,563	20,949,763	19,197,213

Source: Ocuphire Pharma