May 15, 2023

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

End-of-Phase 2 Meeting with FDA Anticipated in 2H 2023 to Confirm Phase 3 Regulatory Path for Oral APX3330 in Diabetic Retinopathy (DR)

PDUFA date for Nyxol First Indication in Reversal of Pharmacologically-Induced Mydriasis (RM) Set for September 28, 2023; Nyxol Development and Commercialization Funded by Viatris

Cash Balance of $39 Million Expected to Fund Operations into 2025

FARMINGTON HILLS, Mich., May 15, 2023 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing novel therapies for the treatment of unmet needs of patients with retinal and refractive eye disorders, today announced financial results for the first quarter ended March 31, 2023, and provided a corporate update.

“Ocuphire made significant progress across both our APX3330 and Nyxol programs throughout the first quarter,” said Rick Rodgers, Interim Chief Executive Officer. “Based on the efficacy and safety data from the ZETA-1 Phase 2 trial of APX3330, we are preparing for an End-of-Phase 2 meeting with the FDA to confirm Phase 3 registration endpoints and study parameters. If approved, APX3330 has the potential to be a non-invasive, oral, early intervention treatment for millions of DR patients at risk of progressing to vision-threatening complications. For Nyxol, we look forward to the September 2023 PDUFA date in its first indication in RM. With a strong cash position and our partner Viatris funding the Nyxol program, we are well positioned to advance both APX3330 and Nyxol programs.”

Key Anticipated Future Milestones

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

Recent Business Highlights

Clinical and Regulatory Updates

- In January 2023, the Company announced topline results from the ZETA-1 Phase 2 trial of oral APX3330 for the treatment of diabetic retinopathy (DR). Oral APX3330 achieved statistical significance on a key pre-specified secondary endpoint of binocular ≥ 3-step worsening of DRSS and demonstrated favorable safety and tolerability. This binocular secondary endpoint is a potential Phase 3 registration endpoint. The Company plans an End-of-Phase 2 FDA meeting in the second half of 2023 to formally confirm this endpoint and other clinical trial parameters.

- In February 2023, the Company announced that the FDA has accepted for review a New Drug Application (NDA) for Nyxol® in RM and set a PDUFA date of September 28, 2023. FDA approval in RM would trigger a $10 million milestone payment to Ocuphire.

- Topline results from the VEGA-2 Phase 3 pivotal trial of Nyxol in presbyopia are expected in late 2023. Nyxol clinical programs in presbyopia and dim light disturbances (DLD) continue to progress as planned.

Presentations, Publications, and Conferences

- From January 2023 through May 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:

    - Results from ZETA-1 Phase 2 trial of APX3330 in DR were presented for the first time to the medical community at the 20th Angiogenesis, Exudation, and Degeneration 2023 Meeting in February 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.
    - Previously announced results from ZETA-1 Phase 2 trial of APX3330 in DR were presented to the medical community at the American Society of Cataract and Refractive Surgery (ASCRS) Annual Meeting in May 2023.

Corporate

In April, Ocuphire announced the appointment of Rick Rodgers as Interim President and Chief Executive Officer. Mr. Rodgers is a seasoned operating executive with 20 years of experience in biopharmaceutical management.

First Quarter ended March 31, 2023 Financial Highlights

As of March 31, 2023, Ocuphire had cash and cash equivalents of approximately $39.0 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 $1.7 million for the three months ended March 31, 2023. There was no revenue recorded in the three months ended March 31, 2022. Revenue

during the first quarter of 2023 was derived from the reimbursement of research and development services under the Nyxol License Agreement.

General and administrative expenses for the three months ended March 31, 2023 were $2.3 million, compared to $1.7 million for the three months ended March 31, 2022. The increase was primarily attributable to an increase in stock-based compensation, professional services fees, legal support, and business development activities, offset in part by a decrease in payroll and insurance costs on a net basis. General and administrative expenses included $0.5 million and $0.3 million in stock-based compensation expense during the three months ended March 31, 2023, and 2022, respectively.

Research and development expenses for the three months ended March 31, 2023 were $5.6 million, compared to $4.8 million for the three months ended March 31, 2022. The increase was primarily attributable to increased manufacturing activities for Nyxol and APX3330 period over period as well as increased payroll and consulting costs during the current period. Research and development expenses also included $0.3 million and $0.1 million in stock-based compensation expense during the three months ended March 31, 2023, and 2022, respectively.

The loss from operations for the quarter ended March 31, 2023 was $6.1 million, compared to $6.5 million for the quarter ended March 31, 2022.

Net loss for the quarter ended March 31, 2023 was $5.8 million or ($0.28) per share, compared to $6.6 million or ($0.35) per share for the quarter ended March 31, 2022.

For further details on Ocuphire's financial results, refer to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2023 to be filed with the Securities and Exchange Commission.

About Ocuphire Pharma

Ocuphire is a publicly traded (Nasdaq: OCUP), clinical-stage, ophthalmic biopharmaceutical company focused on developing novel therapies for the treatment of unmet needs of patients with retinal and refractive eye disorders.

Ocuphire's lead product candidate targeting retinal (back-of-the-eye) indications 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. An End-of-Phase 2 meeting with the FDA is planned for APX3330.

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 (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. 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 timing and success of identifying a permanent CEO, the potential receipt of regulatory approval for Nyxol for the treatment of RM, the ability to fund operations into 2025, the occurrence and timing of an End-of-Phase 2 meeting with the FDA, results of the VEGA-2 Phase 3 trial, the ability of Viatris to execute successful US and global launches of Nyxol, and the ability to determine a path to registration for APX3330. 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	Bret Shapiro CoreIR brets@coreir.com

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

	As of	
	March 31, 2023 (unaudited)	**December 31, 2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 38,988	$ 42,634
Accounts receivable	2,834	1,298
Contract asset	2,467	3,552
Prepaids and other current assets	1,088	1,453
Short-term investments	22	49
Total current assets	45,399	48,986
Property and equipment, net	5	6
Total assets	$ 45,404	$ 48,992
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,221	$ 1,069
Accrued expenses	1,933	1,684
Total current liabilities	4,154	2,753
Warrant liabilities	—	—
Total liabilities	4,154	2,753
Commitments and contingencies		
Stockholders' equity		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of March 31, 2023 and December 31, 2022; no shares issued and outstanding at March 31, 2023 and December 31, 2022.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of March 31, 2023 and December 31, 2022; 20,947,830 and 20,861,315 shares issued and outstanding at March 31, 2023 and December 31, 2022, respectively.	2	2
Additional paid-in capital	118,519	117,717
Accumulated deficit	(77,271)	(71,480)
Total stockholders' equity	41,250	46,239

Total liabilities and stockholders' equity	$ 45,404	$ 48,992

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

	Three Months Ended March 31,	
	2023	**2022**
License and collaborations revenue	$ 1,749	$ —
Operating expenses:		
General and administrative	2,285	1,736
Research and development	5,595	4,772
Total operating expenses	7,880	6,508
Loss from operations	(6,131)	(6,508)
Interest expense	—	(5)
Fair value change in warrant liabilities	—	—
Other income (expense), net	340	(82)
Loss before income taxes	(5,791)	(6,595)
Benefit (provision) for income taxes	—	—
Net loss	(5,791)	(6,595)
Other comprehensive loss, net of tax	—	—
Comprehensive loss	$ (5,791)	$ (6,595)
Net loss per share:		
Basic and diluted	$ (0.28)	$ (0.35)
Number of shares used in per share calculations:		
Basic and diluted	20,939,607	18,888,471

Source: Ocuphire Pharma