November 13, 2023

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

Successful End-of-Phase 2 Meeting with FDA for Oral APX3330; Agreement on Phase 3 Registration Endpoint

RYZUMVI™ Approved by FDA; Ocuphire Received $10 million Regulatory Milestone Payment

VEGA-2 Phase 3 Presbyopia Trial Met Primary Endpoint; Viatris Expected to Continue Phase 3 Development in 1H 2024

George Magrath, M.D., M.B.A., M.S. Named as CEO; Conference Call Scheduled for December 5th, 2023, at 10 AM ET

FARMINGTON HILLS, Mich., Nov. 13, 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 third quarter ended September 30, 2023 and provided a corporate update.

“We achieved important regulatory and clinical milestones in recent months, particularly in advancing oral APX3330 towards a registrational Phase 3 program in diabetic retinopathy,” said Chief Executive Officer George Magrath, M.D., M.B.A., M.S., “We now have agreement with the FDA on the Phase 3 primary endpoint of 3-step worsening (measuring delay or prevention of progression) on a binocular Diabetic Retinopathy Severity Scale, and plan to finalize the protocol and statistical analysis plan with the FDA through a Special Protocol Assessment submission. If approved, APX3330 has the potential to be the first non-invasive, early treatment to delay or prevent vision-threatening complications in millions of patients with non-proliferative DR. We are very pleased to have recently received FDA approval of RYZUMVI™ (phentolamine ophthalmic solution) 0.75% eye drops for the reversal of pharmacologically induced mydriasis and look forward to our partner Viatris’ commercial launch in the first half of 2024. We are also excited to share that the VEGA-2 Phase 3 trial in presbyopia met its primary endpoint, and Viatris is expected to continue the Phase 3 development in the first half of 2024.”

Key Anticipated Future Milestones

- **APX3330:** Special Protocol Assessment (“SPA”) submission and agreement with U.S. Food and Drug Administration (“FDA”)

Recent Business Highlights

Clinical and Regulatory Updates

- In October 2023, Ocuphire had a successful End-of-Phase 2 ("EOP2") meeting with the FDA for oral APX3330 in Diabetic Retinopathy and agreed on the Phase 3 primary endpoint of 3-step worsening (measuring delay or prevention of progression) on a binocular diabetic retinopathy severity scale. The Company plans to submit a SPA to the FDA to agree on the protocol and statistical analysis plan of the first Phase 3 trial.
- In September 2023, Ocuphire and Viatris announced FDA approval of RYZUMVI™ (phentolamine ophthalmic solution) 0.75% for the treatment of pharmacologically induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic agents (e.g., tropicamide). Ocuphire received a $10 million milestone payment from Viatris upon the approval. RYZUMVI is expected to be commercially available in the U.S. in the first half of 2024.
- The VEGA-2 Phase 3 study evaluating phentolamine ophthalmic solution 0.75% in presbyopia achieved its primary endpoint. Viatris is expected to continue Phase 3 development for this indication in the first half of 2024.
- The Company has submitted a SPA to the FDA for night vision disturbances or dim light vision and Viatris is expected to continue Phase 3 development of phentolamine ophthalmic solution 0.75% for this indication in the first half of 2024.

Corporate

On August 10, 2023, Ocuphire entered into a common share purchase agreement with Lincoln Park Capital Fund, LLC ("LPC"). 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. There are no warrants or other share classes associated with the purchase agreement. Proceeds from share sales are expected to fund the future development of APX3330 and to be used for general corporate purposes.

Presentations, Publications, and Conferences

Beginning in the third quarter of 2023, several presentations at medical meetings featured previously announced data from the ZETA-1 Phase 2 trial of APX3330 in diabetic retinopathy. Highlights include:

- A presentation at the Eyecelerator American Academy of Ophthalmology 2023 Retina Showcase by Jay Pepose, M.D. Ph.D., Chief Medical Advisor, highlighting the agreement of Phase 3 registration endpoint at the EOP2 FDA meeting.
- A paper presentation delivered by Veeral Sheth, M.D., at the 23rd Euretina Congress in October 2023.
- An oral presentation delivered by Anat Lowenstein, M.D., at the 56th Annual Retina

Society Scientific Meeting in October 2023.

- An oral presentation delivered by Priya Vakharia, M.D., at the Women in Ophthalmology Summer Symposium in August 2023. The abstract was rated as one of the top three scoring abstracts out of nearly 600 submissions and received the Joanne Angle Abstract of Distinction Award.

Third Quarter ended September 30, 2023, Financial Highlights

As of September 30, 2023, Ocuphire had cash and cash equivalents of approximately $42.4 million. Based on current projections, management believes the present cash on hand will be sufficient to fund operations into 2025.

License and collaborations revenue was $11.9 million and $17.4 million for the three and nine months ended September 30, 2023, respectively. There was no license and collaborations revenue during the three and nine months ended September 30, 2022. Revenue during the three and nine months ended September 30, 2023, was derived from the achievement of a $10.0 million milestone attributed to the FDA's approval of RYZUMVI™ for reversal of mydriasis and from the reimbursement of research and development services under the License Agreement with Viatris in the amount of $1.9 and $7.4 million, respectively.

General and administrative expenses for the three and nine months ended September 30, 2023, were $2.1 million and $8.7 million, respectively, compared to $1.7 million and $5.2 million, respectively, for the three and nine months ended September 30, 2022. The increases from the comparable periods in 2022 were attributable to professional services and personnel related and other costs. General and administrative expenses included stock-based compensation expenses.

Research and development expenses for the three and nine months ended September 30, 2023, were $3.5 million and $13.8 million, respectively, compared to $2.8 million and $10.8 million, respectively, for the three and nine months ended September 30, 2022. The increases from the comparable periods in 2022 were primarily attributable to increased drug manufacturing, toxicology services and payroll and consulting related costs. Research and development expenses also included stock-based compensation expenses.

Income (loss) from operations for the three and nine months ended September 30, 2023, was $6.4 million and ($5.1) million, respectively, compared ($4.5) million and ($16.0) million, respectively, for the three and nine months ended September 30, 2022.

Net income (loss) for the three and nine months ended September 30, 2023, was $5.6 million and ($5.2) million, respectively, compared to ($4.5) million and ($16.1) million, respectively, for the three and nine months ended September 30, 2022. Basic net income (loss) per share for the three and nine months ended September 30, 2023, was $0.26 and ($0.25) per share, respectively, compared to ($0.22) and ($0.82) per share, respectively, for the three and nine months ended September 30, 2022.

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

Conference Call and Webcast Details:

Date: December 5th, 2023
Time: 10:00 AM ET
Dial-in information: 1-877-407-4018 (US); 1-201-689-8471 (International); Call me™
Passcode: 13742669
Webcast link

Participants can use Guest dial-in #s above and be answered by an operator OR click the Call me™ link for instant telephone access to the event. Call me™ link will be made active 15 minutes prior to scheduled start time.

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 the transcription factors 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 deplete VEGF below the levels required for normal function. 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 recently been completed, and a Special Protocol Assessment is planned to be submitted with the U.S. Food and Drug Administration (FDA).

DR affects approximately 10 million people with diabetes and is projected to impact over 14 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 non-existent 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 treatment burden of the frequent eye injections 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 U.S. 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, and geographic atrophy. 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 phentolamine ophthalmic solution 0.75%. Phentolamine is a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size by uniquely blocking the alpha-1 receptors found on the iris dilator muscle without affecting the ciliary muscle. In September 2023, the FDA approved RYZUMVI™ (phentolamine ophthalmic solution 0.75%) to treat pharmacologically induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic agents (e.g., tropicamide). Phentolamine ophthalmic solution 0.75% is also in Phase 3 clinical development for the treatment of presbyopia and dim light (night) vision disturbances.

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, study parameters for Phase 3 pivotal studies, Phase 3 development, FDA agreement on Special Protocol Assessment, the potential for APX330 to be the first non-invasive, early treatment to delay or prevent progression to vision-threatening complications, ability to fund operations into 2025, and the commercialization of RYZUMVI™. 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 phentolamine ophthalmic solution 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

George Magrath, M.D., M.B.A., M.S. 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	
	September 30, 2023 (unaudited)	**December 31, 2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 42,350	$ 42,634
Accounts receivable	10,132	1,298
Contract assets and unbilled receivables	1,211	3,552
Prepaids and other current assets	484	1,453
Short-term investments	11	49
Total current assets	54,188	48,986
Property and equipment, net	3	6
Total assets	$ 54,191	$ 48,992
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,890	$ 1,069
Accrued expenses	1,926	1,684
Derivative liability	93	—
Total current liabilities	3,909	2,753
Total liabilities	3,909	2,753
Commitments and contingencies		
Stockholders' equity		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of September 30, 2023 and December 31, 2022; no shares issued and outstanding at September 30, 2023 and December 31, 2022.	—	—

Common stock, par value $0.0001; 75,000,000 shares authorized as of September 30, 2023 and December 31, 2022; 22,610,131 and 20,861,315 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively.	2	2
Additional paid-in capital	126,951	117,717
Accumulated deficit	(76,671)	(71,480)
Total stockholders' equity	50,282	46,239
Total liabilities and stockholders' equity	$ 54,191	$ 48,992

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

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
License and collaborations revenue	$ 11,935	$ —	$ 17,358	$ —
Operating expenses:				
General and administrative	2,055	1,703	8,680	5,215
Research and development	3,494	2,835	13,812	10,769
Total operating expenses	5,549	4,538	22,492	15,984
Income (loss) from operations	6,386	(4,538)	(5,134)	(15,984)
Financing costs	(1,328)	—	(1,328)	—
Interest expense	—	—	—	(9)
Fair value change in derivative liability	61	—	61	—
Other income (expense), net	456	7	1,224	(60)
Income (loss) before income taxes	5,575	(4,531)	(5,177)	(16,053)
Provision for income taxes	(14)	—	(14)	—
Net income (loss)	5,561	(4,531)	(5,191)	(16,053)
Other comprehensive income (loss), net of tax	—	—	—	—
Comprehensive income (loss)	$ 5,561	$ (4,531)	$ (5,191)	$ (16,053)
Net income (loss) per share:				
Basic	$ 0.26	$ (0.22)	$ (0.25)	$ (0.82)
Diluted	$ 0.25	$ (0.22)	$ (0.25)	$ (0.82)
Number of shares used in per share calculations:				

Basic	21,446,648	20,498,229	21,117,211	19,635,651
Diluted	22,405,995	20,498,229	21,117,211	19,635,651

Source: Ocuphire Pharma