August 13, 2024

Ocuphire Pharma Announces Financial Results for Second Quarter 2024 and Provides Corporate Update

VEGA-3 Phase 3 Study of Phentolamine Ophthalmic Solution in Presbyopia is Recruiting Patients with Top-Line Data Expected in 2025

LYNX-2 Phase 3 Study of Phentolamine Ophthalmic Solution Continues Enrollment with Top-Line Data Expected in 2025

Preparatory Steps Towards Phase 2/3 with APX3330 in Diabetic Retinopathy are Ongoing

Cash Position of $41.4 million Provides Runway Anticipated into mid-2025

FARMINGTON HILLS, Mich., Aug. 13, 2024 (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 patients with retinal and refractive eye disorders, today announced financial results for the second quarter ended June 30, 2024, and provided a corporate update.

“APX3330 has potential to be the first non-invasive, early treatment to delay vision-threatening complications in millions of patients with non-proliferative diabetic retinopathy (“DR”) who otherwise remain untreated, and we are pleased with the progress on our preparations for the next clinical study. We continue to work collaboratively with the U.S. Food and Drug Administration (the “FDA”) on our submitted Special Protocol Assessment (“SPA”) for Phase 2/3 and the overall clinical development plan for APX3330,” said George Magrath, M.D., M.B.A., M.S., CEO of Ocuphire.

Dr. Magrath continued, “We are also making progress on additional indications for phentolamine ophthalmic solution. The VEGA-3 Phase 3 trial in presbyopia, funded by our commercial partner, has begun recruiting. Phentolamine ophthalmic solution has demonstrated a differentiated product profile in prior presbyopia studies, and we believe it could be a promising and convenient treatment option for the growing number of people with this condition. The LYNX-2 Phase 3 study in dim light disturbances (“DLD”) has seen strong enrollment and could provide a differentiated product for patients.”

Clinical and Regulatory Updates

APX3330

- Data from studies with APX3330 in DR were featured at multiple medical meetings throughout the second quarter, including at the Association for Research in Vision and Ophthalmology Special Interest Group panel, the American Society of Retina

Specialists 42nd Annual Scientific Meeting, the Clinical Trials at the Summit meeting and the Retinal Imaging Biomarkers & Endpoints Summit meeting. Presentations featured results from a subset analysis that evaluated the efficacy of APX3330 in slowing DR progression using a binocular DR severity person-level scale in high-risk non-proliferative DR patients.

- Discussions with the FDA are ongoing regarding the SPA and the development pathway of APX3330 for the treatment of DR.

Phentolamine Ophthalmic Solution 0.75%

- The VEGA-3 Phase 3 trial evaluating phentolamine ophthalmic solution 0.75% for the treatment of presbyopia is now recruiting. This randomized, double-masked, placebo-controlled, multi-center Phase 3 trial is expected to enroll up to 545 subjects with presbyopia at up to 40 U.S. sites. Participants will be randomized 3:2 to receive phentolamine ophthalmic solution 0.75% or placebo. The primary endpoint is the percentage of subjects with 15-letter improvement in photopic binocular distance-corrected near visual acuity (DCNVA) on the eighth day following their first visit. Topline results are expected in 2025.
- Enrollment has been strong for the LYNX-2 Phase 3 registration study evaluating phentolamine ophthalmic solution 0.75% for the treatment of decreased visual acuity under low (mesopic) light conditions following keratorefractive surgery. The LYNX-2 trial is being conducted under conditions of an SPA with the FDA.
- The Company is also planning an additional Phase 3 study for decreased vision under mesopic (low) light conditions following keratorefractive surgery, LYNX-3, in 2024.
- The phentolamine ophthalmic solution 0.75% development portfolio is being funded by Ocuphire's partner in both indications (presbyopia and dim light vision disturbances).

Financial Highlights for the Second Quarter Ended June 30, 2024

As of June 30, 2024, Ocuphire had cash and cash equivalents of $41.4 million. Based on current projections, management believes that the cash on hand will be sufficient to fund operations for the next twelve months.

License and collaborations revenue was $1.1 million and $3.7 million for the three months ended June 30, 2024 and 2023, respectively. Revenue during both quarterly periods was derived from the commercial partner License Agreement largely for the reimbursement of research and development services. Revenue for the second quarter includes an earned royalty payment in the amount of $19,000 from the sales of RYZUMVI™, indicated for the treatment of pharmacologically-induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents, by our commercial partner.

General and administrative expenses for the three months ended June 30, 2024 were $3.4 million compared to $4.3 million for the three months ended June 30, 2023. The decrease was primarily attributable to a net reduction in payroll-related costs and a reduction in other operating expenses. Partially offsetting these reductions were increases in business development costs, legal support costs and other operating expenses, compared to the corresponding prior year period. General and administrative expenses included $0.5 million and $1.2 million in stock-based compensation expense during the three months ended June

30, 2024 and 2023, respectively.

Research and development expenses for the three months ended June 30, 2024 were $6.1 million compared to $4.7 million for the three months ended June 30, 2023. The increase was primarily attributable to increased costs related to manufacturing and toxicology costs for APX3330, payroll related costs and other operating costs, offset in part by decreased clinical costs attributed to the phentolamine ophthalmic solution 0.75% VEGA-2 trial when compared to the corresponding prior year period. Pursuant to the commercial partner License Agreement, our budgeted research and development expenses related to the development of phentolamine ophthalmic solution 0.75% are fully reimbursed by our commercial partner. Research and development expenses also included $0.3 million in stock-based compensation expense during each of the three months ended June 30, 2024 and 2023.

Net loss for the quarter ended June 30, 2024, was $7.8 million or $(0.30) per basic and diluted share as compared to a net loss of $5.0 million or $(0.24) per basic and diluted share for the second quarter of 2023.

For further details on Ocuphire's financial results, including results for the six-month period ended June 30, 2024, refer to the Company's Quarterly Report on Form 10-Q to be filed with the Securities and Exchange Commission.

About Ocuphire Pharma

Ocuphire Pharma, Inc. (Nasdaq: OCUP) is a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing novel therapies for the treatment of patients with retinal and refractive eye disorders. Ocuphire's lead product candidate, APX3330, a novel small-molecule inhibitor of Ref-1 (reduction oxidation effector factor-1 protein), is in development for diabetic retinopathy. In addition, Ocuphire's late-stage product candidate Phentolamine Ophthalmic Solution 0.75%, a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size, is being developed for presbyopia and dim light vision disturbances and is currently approved and marketed as RYZUMVI™, indicated for the treatment of pharmacologically-induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents, by our commercial partner. 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 applications of phentolamine ophthalmic solution 0.75% in ophthalmology, the registration program for phentolamine ophthalmic solution 0.75%, the LYNX-2 Phase 3 registration study, the efficacy of APX3330 in slowing the progression of diabetic retinopathy, the safety and tolerability of APX3330, logistical details regarding the upcoming VEGA-3 Phase 3 clinical trial and estimates of when data will become available from such trial, the benefits, uses and side effects of phentolamine ophthalmic solution 0.75% treatment, ongoing discussions with the U.S. Food and Drug Administration regarding our drug products, continued drug development under our agreement with our commercial partner, and the sufficiency and amount of cash on hand to meet future funding needs.

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 and in subsequent filings with the U.S. Securities and Exchange Commission. 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,” “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 our commercial partner, 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 SEC 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.

Contacts

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

	As of	
	June 30, 2024	**December 31, 2023**
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 41,409	$ 50,501
Accounts receivable	1,358	926
Contract assets and unbilled receivables	948	1,407
Prepaids and other assets	1,114	1,099
Short-term investments	5	15
Total current assets	44,834	53,948
	—	—
Property and equipment, net		
Total assets	$ 44,834	$ 53,948
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 634	$ 2,153
Accrued expenses	3,490	1,815
Derivative liability	74	74
Total current liabilities	4,198	4,042
Total liabilities	4,198	4,042

Commitments and contingencies		
Stockholders' equity:		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of June 30, 2024 and December 31, 2023; no shares issued and outstanding at June 30, 2024 and December 31, 2023.	—	—
Common stock, par value $0.0001; 125,000,000 and 75,000,000 shares authorized as of June 30, 2024 and December 31, 2023, respectively; 25,979,038 and 23,977,491 shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively.	3	2
Additional paid-in capital	136,970	131,370
Accumulated deficit	(96,337)	(81,466)
Total stockholders' equity	40,636	49,906
Total liabilities and stockholders' equity	$ 44,834	$ 53,948

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,	
	2024	**2023**	**2024**	**2023**
License and collaborations revenue	$ 1,112	$ 3,674	$ 2,823	$ 5,423
Operating expenses:				
General and administrative	3,354	4,340	8,024	6,625
Research and development	6,086	4,723	10,835	10,318
Total operating expenses	9,440	9,063	18,859	16,943
Loss from operations	(8,328)	(5,389)	(16,036)	(11,520)
Other income, net	563	428	1,165	768
Loss before income taxes	(7,765)	(4,961)	(14,871)	(10,752)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(7,765)	(4,961)	(14,871)	(10,752)
Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (7,765)	$ (4,961)	$ (14,871)	$ (10,752)
Net loss per share:				
Basic and diluted	$ (0.30)	$ (0.24)	$ (0.59)	$ (0.51)

Number of shares used in per share calculations:				
Basic and diluted	25,827,265	20,959,807	25,175,596	20,949,763

Source: Ocuphire Pharma