May 10, 2024

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

FARMINGTON HILLS, Mich., May 10, 2024 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing small molecule therapies for the treatment of patients with retinal and refractive eye disorders, today announced financial results for the first quarter ended March 31, 2024, and provided a corporate update.

“Ocuphire has made significant progress in 2024, with important developments for both APX3330 and RYZUMVI™,” said George Magrath, M.D., M.B.A., M.S., CEO of Ocuphire. “We have been engaged in productive dialogue with the U.S. Food and Drug Administration (FDA) with respect to our submitted Special Protocol Assessment (SPA) to formalize the protocol and statistical analysis plan for future Phase 2/3 registrational trials of APX3330 in diabetic retinopathy (DR). If APX3330 is approved, we believe it could be a promising oral treatment option for slowing disease progression in patients with non-proliferative DR who otherwise are monitored and untreated until they progress to sight-threatening disease. The recent commercial launch of RYZUMVI™ by our partner Viatris, Inc. (Viatris) (NASDAQ: VTRS) was a major milestone, and an important validation of the clinical development work conducted by the Ocuphire team over the past several years to advance this product and secure FDA approval. Viatris now has the opportunity to create further value as it pursues additional indications for phentolamine ophthalmic solution, including the treatment of decreased low contrast visual acuity under low light conditions as well as presbyopia.”

Clinical and Regulatory Updates

APX3330

- In February 2024, Ocuphire submitted a Special Protocol Assessment (SPA) to the U.S. Food and Drug Administration (FDA) to seek agreement on the clinical trial protocol and statistical analysis plan for a Phase 2/3 registration study for APX3330 in DR. This request followed an End-of-Phase 2 meeting held with the FDA late in 2023, during which the company aligned on the registrational primary endpoint of 3-step or more worsening on a binocular Diabetic Retinopathy Severity Scale (DRSS) person-level scale. Dialogue with the FDA is ongoing, and specifics on the study design and the anticipated timing will be announced if and when an agreement is reached with the FDA.
- Earlier this month, a subset analysis from the ZETA-1 trial was presented at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting, which took place in Seattle, Washington. This analysis evaluated the efficacy of APX3330 in

slowing DR progression on the target population of the planned Phase 2/3 study, and the FDA agreed upon registration endpoint of a 3-step change on a binocular DRSS person-level scale, which is considered clinically meaningful. The presented results in addition to further analyses demonstrated an enhanced treatment effect for APX3330 in this target population.

- In conjunction with this update, a subpopulation analysis of ZETA-1 data in NPDR will be released.

Phentolamine Ophthalmic Solution

- In April 2024, Ocuphire's partner Viatris launched RYZUMVI™ (phentolamine ophthalmic solution) for the treatment of pharmacologically-induced mydriasis in the U.S. in adult and pediatric patients aged 3 and older. Ocuphire has a global license agreement with Viatris to co-develop and commercialize Phentolamine Ophthalmic Solution 0.75% (PS). Under the terms of this agreement, Ocuphire is now recognizing royalties on commercial sales and may be eligible to receive future commercial milestones. For more information on the commercial launch, refer to the announcement on Viatris' corporate website here.
- In April 2024, the first subject was enrolled in the LYNX-2 Phase 3 registration study evaluating PS 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 a SPA with the FDA. As previously announced, Ocuphire received written agreement from the FDA that the clinical trial protocol and planned statistical analysis of the LYNX-2 Phase 3 trial would adequately address objectives supporting regulatory submission and a potential future marketing application in this indication.

Corporate Updates

- In February 2024, Ocuphire appointed Nirav Jhaveri, C.F.A, M.B.A., as Chief Financial Officer and Ashwath (Ash) Jayagopal, Ph.D., M.B.A., as Chief Scientific and Development Officer.

Financial Highlights for the First Quarter Ended March 31, 2024

As of March 31, 2024, Ocuphire had cash and cash equivalents of $47.2 million. Based on current projections, management believes that the cash on hand will be sufficient to fund operations into mid-2025.

License and collaborations revenue was $1.7 million for first quarter ending March 31, 2024 compared with $1.7 million in the first quarter of 2023. Revenue during both quarterly periods was derived from the Viatris license agreement largely for the reimbursement of research and development services. During the first quarter of 2024 Ocuphire earned its first royalty payment in the amount of $3,000 stemming from the sale of RYZUMVI by Viatris in late March 2024.

General and administrative expenses for the first quarter ended March 31, 2024 were $4.7 million, compared to $2.3 million for first quarter of 2023. The increase period over period was primarily attributable to increases in payroll related costs, stock-based compensation, professional services, corporate legal support, legal fees associated with intellectual

property, business development activities and general operating costs. General and administrative expenses included $0.8 million and $0.5 million in stock-based compensation expense during the quarters ended March 31, 2024 and 2023, respectively.

Research and development expenses for the first quarter ended March 31, 2024 were $4.7 million, compared to $5.6 million for the first quarter of 2023. The decrease was primarily attributable to lower clinical costs, lower regulatory costs, and lower manufacturing expenses. These were offset in part by increases in toxicology costs, payroll costs, and general consulting costs. Pursuant to the Viatris license agreement, Ocuphire's budgeted research and development expenses related to the development of PS Products have been fully reimbursed by Viatris to date. Research and development expenses included $0.2 million and $0.3 million in stock-based compensation expense during the quarters ended March 31, 2024 and 2023, respectively.

Net loss for the quarter ended March 31, 2024 was $7.1 million (or $0.29 per basic and diluted share) as compared to a net loss of $5.8 million (or $0.28 per basic and diluted share) for the first quarter of 2023.

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

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 SPA was submitted to the U.S. Food and Drug Administration (FDA) in February 2024 and active discussions continue with the agency.

In addition, Ocuphire has a partnership with Viatris to develop and commercialize 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 research takeaways from the ZETA-1 trial, the efficacy of APX3330 in slowing the progression of diabetic retinopathy, the safety and tolerability of APX3330, applications of PS in ophthalmology, the registration program for PS, the LYNX-2 Phase 3 registration study, the benefits, uses, and side effects of PS treatment, ongoing discussions with the FDA regarding various of our drug products, continued drug development under our agreement with Viatris, and the sufficiency 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 filing with the U.S. Securities and Exchange Commission (SEC). 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 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 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	
	March 31, 2024	**December 31, 2023**
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 47,161	$ 50,501
Accounts receivable	1,924	926
Contract assets and unbilled receivables	1,194	1,407
Prepaids and other assets	1,560	1,099
Short-term investments	5	15
Total current assets	51,844	53,948
Property and equipment, net	—	—
Total assets	$ 51,844	$ 53,948

Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,064	$ 2,153
Accrued expenses	3,649	1,815
Derivative liability	74	74
Total current liabilities	5,787	4,042
Total liabilities	5,787	4,042
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of March 31, 2024 and December 31, 2023; no shares issued and outstanding at March 31, 2024 and December 31, 2023.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of March 31, 2024 and December 31, 2023; 25,085,592 and 23,977,491 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively.	3	2
Additional paid-in capital	134,626	131,370
Accumulated deficit	(88,572)	(81,466)
Total stockholders' equity	46,057	49,906
Total liabilities and stockholders' equity	$ 51,844	$ 53,948

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

	Three Months Ended March 31,	
	2024	**2023**
License and collaborations revenue	$ 1,711	$ 1,749
Operating expenses:		
General and administrative	4,670	2,285
Research and development	4,749	5,595
Total operating expenses	9,419	7,880
Loss from operations	(7,708)	(6,131)
Fair value change in derivative liabilities	—	—
Other income, net	602	340

Loss before income taxes	(7,106)	(5,791)
Benefit (provision) for income taxes	—	—
Net loss	(7,106)	(5,791)
Other comprehensive loss, net of tax	—	—
Comprehensive loss	$ (7,106)	$ (5,791)
Net loss per share:		
Basic and diluted	$ (0.29)	$ (0.28)
Number of shares used in per share calculations:		
Basic and diluted	24,520,475	20,939,607

Source: Ocuphire Pharma