May 13, 2022

Ocuphire Pharma Announces Financial Results for First Quarter 2022 and Provides Corporate Update

Nyxol Posts 5 Positive Clinical Data Readouts Including Presbyopia in Last 12 Months

Successfully Enrolled 4 Late-Stage Trials for Nyxol and APX3330 in First Quarter

NDA Filing on Track for Late 2022 for Potential 2023 Approval of Nyxol as Only Dilation Reversal Drop

Data Expected in 2H22 from Phase 2b Trial of Oral APX3330 for Diabetic Retinopathy

FARMINGTON HILLS, Mich., May 13, 2022 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of refractive and retinal eye disorders, today announced financial results for the first quarter ended March 31, 2022 and provided a corporate update.

“We have kicked off 2022 with a high level of productivity and execution resulting in multiple positive clinical data readouts across our late-stage programs,” said Mina Sooch, MBA, founder and CEO of Ocuphire Pharma. “Year to date, we presented at 10 conferences and met with many KOLs, reinforcing the growing awareness and enthusiasm for our programs, particularly among doctors anticipating an eye drop treatment to reverse dilation. We recently reported positive data from the MIRA-3 and MIRA-4 trials, marking completion of the clinical activities to support the planned NDA filing for Nyxol in the Reversal of Mydriasis (RM) indication later this year. In our retinal program, we look forward to reporting top-line Phase 2b data in the second half of the year for APX3330, an novel oral treatment option for the large unmet need of over 7 million diabetic retinopathy patients who are generally asymptomatic with a progressive vision-threatening disease and are not routinely treated with approved anti-VEGF injections. With the approval of the first artificial-intelligence-based screening of diabetic retinal diseases, we expect an increase in the identification of DR patients.”

Key Anticipated Future Milestones

- **Reversal of Mydriasis (RM):** Planned New Drug Application (NDA) with the FDA for Nyxol in RM indication in late 2022, with potential launch as first dilation reversal drop in 2H 2023

- **Presbyopia:** Initiate VEGA Phase 3 program in mid-2022 investigating Nyxol alone and Nyxol with 0.4% low-dose pilocarpine (LDP) as adjunctive therapy and, if

successful, expect to file an NDA in 2023

- **Night Vision Disturbances (NVD):** Report top-line results from the Nyxol Phase 3 LYNX-1 trial in 2Q 2022

- **Diabetic Retinopathy (DR) and Diabetic Macular Edema (DME):**Report top-line results from the APX3330 Phase 2b ZETA-1 trial in 2H 2022

First Quarter and Recent Business Highlights

Clinical Development

- In January, the Company announced new positive data from the VEGA-1 Phase 2 trial for Nyxol as a single agent in presbyopia, showing that one drop of Nyxol had statistically significant improvement in 3 lines of near vision efficacy at 12-hours compared to placebo.

- In January, the Company completed enrollment of the LYNX-1 Phase 3 Trial investigating Nyxol for the treatment of night vision disturbances in 145 patients (target of 140).

- In February, Ocuphire held a Type-C meeting with the FDA from which it obtained guidance regarding the design of pivotal studies for filing an NDA to seek approvals of Nyxol for the treatment of presbyopia, both as a single agent and with LDP as adjunct therapy eye drops.

- In March, the Company announced successful results from the MIRA-3 Phase 3 registration trial of Nyxol for RM, demonstrating significant and rapid reversal of mydriasis. In addition, multiple key secondary endpoints met statistical significance, including early onset of action, durable response over 24 hours, similar efficacy with one or two drops, and efficacy regardless of iris color or mydriatic agent used.

- In March, the Company completed enrollment of 103 (target of 90-100) diabetic retinopathy patients in the ZETA-1 Phase 2b trial of first-in-class oral APX3330. Masked safety data from the trial, first announced during the R&D Day event in January 2022 and later presented through May at medical conferences, demonstrated a favorable safety profile, consistent with prior studies.

- In April, the Company completed the last clinical trial supporting a planned NDA submission with the announcement of positive results from the MIRA-4 Phase 3 pediatric study evaluating Nyxol for RM. The study met its primary safety endpoint, demonstrating a favorable safety and tolerability profile with no adverse events reported.

Presentations, Publications, and Conferences

- In January through May 2022, Ocuphire was represented at conferences by Mina Sooch and several prominent key thought leaders, including David Boyer, MD, David Lally, MD, Jay Pepose, MD, Inder Paul Singh, MD, Douglas Devries, OD, and James

Katz, MD, who presented updates on Nyxol in Presbyopia and RM, as well as masked safety data for APX3330 in DR. In total, 16 papers, posters, and panel talks were presented across 10 medical and industry conferences.

Corporate

- In January, the Company held an Investor R&D Day webinar that featured six ophthalmic Key Opinion Leaders: Jay Pepose, MD, PhD, James Katz, MD and Mitchell Jackson, MD from refractive surgery, Paul Karpecki, OD from optometry, and David Boyer, MD, and Peter Kaiser, MD, from retina practice areas who discussed the unmet needs in RM, presbyopia and DR addressed by Ocuphire's two late-stage clinical drug assets, Nyxol and APX3300. A replay of the event can be found on the Company's corporate website here.

- In March, the Company appointed Jay Pepose, MD, PhD, as its Chief Medical Advisor.

First Quarter Ended March 31, 2022 Financial Highlights

As of March 31, 2022, Ocuphire had cash and cash equivalents of approximately $19.2 million. Based on current projections, management believes the current cash on hand will be sufficient to fund operations into the second quarter of 2023. Cash and cash equivalents as of March 31, 2022 was $5.3 million lower than on December 31, 2021.

General and administrative expenses were $1.7 million for each of the three months ended March 31, 2022 and March 31, 2021.

Research and development expenses for the three months ended March 31, 2022 were $4.8 million compared to $3.5 million for the three months ended March 31, 2021. The $1.3 million increase was primarily attributable to an increased activity level associated with clinical trials and manufacturing activities for Nyxol and APX3330 period over period as well as additional preclinical and other development activities during the current period.

The loss from operations for the quarter ended March 31, 2022 was $6.5 million, compared to $5.2 million for the quarter ended March 31, 2021.

Net loss for the quarter ended March 31, 2022 was $6.6 million or ($0.35) per share, compared to $39.0 million or ($3.57) per share for the quarter ended March 31, 2021 which included a non-cash fair value change in warrant liabilities of $33.8 million.

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, 2022 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 and commercializing therapies for the treatment of several eye disorders. Ocuphire's pipeline currently includes two small-molecule product candidates targeting refractive and retinal indications. The company's lead product candidate, Nyxol® eye drops (0.75% phentolamine ophthalmic solution) is a once-

daily, preservative-free eye drop formulation of phentolamine mesylate, a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size, and is being developed for several indications, including reversal of pharmacologically-induced mydriasis (RM), presbyopia and dim light or night vision disturbances (NVD), and has been studied in 11 completed clinical trials. Ocuphire has reported positive data from MIRA-2 and MIRA-3 registration trials and MIRA-4 pediatric safety trial for the treatment of RM. Ocuphire also reported positive top-line data from a Phase 2 trial of Nyxol for treatment of presbyopia, both Nyxol as a single agent and Nyxol with 0.4% low-dose pilocarpine (LDP) as adjunctive therapy. The company recently completed enrollment in its Phase 3 trial of Nyxol for NVD (LYNX-1). Ocuphire's second product candidate, APX3330, is an oral tablet designed to inhibit angiogenesis and inflammation pathways relevant to retinal and choroidal vascular diseases, such as diabetic retinopathy (DR) and diabetic macular edema (DME) and has been studied in 11 Phase 1 and 2 trials. The company recently announced the completion of enrollment in a Phase 2b clinical trial of APX3330 to treat DR/DME (ZETA-1). Please visit www.clinicaltrials.gov to learn more about Ocuphire's recently completed Phase 3 registration trial in RM (NCT05134974), pediatric safety study in RM (NCT05223478), Phase 3 registration trial in NVD (NCT04638660), and Phase 2b trial in DR/DME (NCT04692688). Ocuphire previously completed the first Phase 3 registration trial in RM (NCT04620213) and Phase 2 trial in presbyopia (NCT04675151). As part of its strategy, Ocuphire will continue to explore opportunities to acquire additional ophthalmic assets and to seek strategic partners for late-stage development, regulatory preparation, and commercialization of drugs in key global markets. 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, the success and timing of planned regulatory filings (including NDA filings), potential increase in the identification of DR patients, future clinical trials, and commercialization of Ocuphire's product candidates. 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) the success and timing of commercialization of any of Ocuphire's product candidates and (x) 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.

Ocuphire Contacts

Mina Sooch, President & CEO
Ocuphire Pharma, Inc.
ir@ocuphire.com
www.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	
	March 31, 2022	**December 31, 2021**
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 19,246	$ 24,534
Prepaids and other current assets	1,095	1,314
Short-term investments	135	219
Total current assets	20,476	26,067
Property and equipment, net	9	10
Total assets	$ 20,485	$ 26,077
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,579	$ 1,584
Accrued expenses	1,419	1,733
Short-term loan	215	538
Total current liabilities	3,213	3,855
Warrant liabilities	—	—
Total liabilities	3,213	3,855
Commitments and contingencies		
Stockholders' equity		

Preferred stock, par value $0.0001; 10,000,000 shares authorized as of March 31, 2022 and December 31, 2021; no shares issued and outstanding at March 31, 2022 and December 31, 2021.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of March 31, 2022 and December 31, 2021; 19,213,651 and 18,845,828 shares issued and outstanding at March 31, 2022 and December 31, 2021, respectively.	2	2
Additional paid-in capital	113,233	111,588
Accumulated deficit	(95,963)	(89,368)
Total stockholders' equity	17,272	22,222
Total liabilities and stockholders' equity	$ 20,485	$ 26,077

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

	Three Months Ended March 31,	
	2022	**2021**
Operating expenses:		
General and administrative	$ 1,736	$ 1,704
Research and development	4,772	3,482
Total operating expenses	6,508	5,186
Loss from operations	(6,508)	(5,186)
Interest expense	(5)	—
Fair value change in warrant liabilities	—	(33,829
Other (expense) income, net	(82)	1
Loss before income taxes	(6,595)	(39,014)
Benefit (provision) for income taxes	—	—
Net loss	(6,595)	(39,014)
Other comprehensive loss, net of tax	—	—
Comprehensive loss	$ (6,595)	$ (39,014)
Net loss per share:		
Basic and diluted	$ (0.35)	$ (3.57)
Number of shares used in per share calculations:		
Basic and diluted	18,888,471	10,923,651

Source: Ocuphire Pharma