August 12, 2022

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

Completed Final Clinical Trials and Pre-NDA FDA Meeting to Support Registration of Nyxol in Reversal of Mydriasis (RM), On Track for Late 2022 NDA Submission and Potential 2023 Approval

Positive Results from Phase 3 LYNX-1 Trial of Nyxol in Night Vision Disturbances (NVD) Mark Sixth Positive Nyxol Clinical Data Readout Across Multiple Indications

Data Expected in 2H 2022 from Phase 2b Trial of APX3330, a Potential First-in-Class Oral Treatment for Diabetic Retinopathy

Plans to Initiate VEGA Phase 3 FDA Registration Program for Nyxol Alone and in Combination with Low-Dose Pilocarpine (LDP) in Presbyopia in 2H 2022

FARMINGTON HILLS, Mich., Aug. 12, 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 second quarter ended June 30, 2022 and provided a corporate update.

“Ocuphire has continued to deliver in the second quarter of 2022 on execution as a highly-productive and capital efficient company, with continued success in late-stage clinical trials, multiple positive data presentations featured at medical meetings, and additional IP protection granted for both Nyxol and APX3330,” said Mina Sooch, MBA, founder and CEO of Ocuphire Pharma. “With the completion of the final clinical trials of Nyxol in reversal of mydriasis, we remain focused on a late 2022 NDA filing as we ramp up our pre-commercial activities and entertain discussions with commercial partners in preparation for the anticipated approval of Nyxol as the first and only dilation reversal drop in 2023. We look forward to reporting top-line results in the second half of this year from the Phase 2b trial of APX3330 in diabetic retinopathy (DR). The opportunity in retina has multi-billion dollar potential. We are well positioned to deliver on our upcoming late-stage clinical and regulatory milestones through the second half of 2022.”

Dr. Jay Pepose, Ocuphire’s Chief Medical Advisor and Board Member added, “A disruptive catalyst later this year will be top-line results from the ongoing multi-center, randomized, double-masked, placebo-controlled Phase 2b ZETA-1 trial evaluating APX3330 in diabetic retinopathy. APX3330 is a novel oral therapy with a dual mechanism of action in validated pathways, decreasing both abnormal angiogenesis and inflammation. It has shown a favorable safety profile for a systemic oral drug in hundreds of patients in 11 prior clinical trials. More recently, masked safety data from 70% of the 103 enrolled patients who

completed 24 weeks of treatment in ZETA-1 trial were presented in July confirming the favorable safety and tolerability profile seen in prior trials. If approved, APX3330 has the potential to be a convenient oral treatment for diabetic patients with non-proliferative diabetic retinopathy and other diabetes-related complications. This could represent a paradigm shift from observation and monitoring progression today to an early, non-injection treatment option."

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:** Plan to initiate VEGA-2 trial, the first of two Phase 3 registration trials intended to support the use of Nyxol alone and Nyxol with 0.4% low dose pilocarpine (LDP) in presbyopia, in 2H of 2022; if successful, the Company expects to file a supplemental NDA for Nyxol as a single-agent for presbyopia and a new NDA for the combination thereafter

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

Second Quarter and Recent Business Highlights

Clinical Development

- In April, the Company announced positive results from the MIRA-4 Phase 3 pediatric study in subjects aged 3-12 years, the final clinical trial supporting a planned NDA submission with for Nyxol in RM. The study met its primary safety endpoint, demonstrating a favorable safety and tolerability profile with no adverse events reported with similar efficacy seen in adults.

- In May, the Company announced positive top-line results from the LYNX-1 Phase 3 trial evaluating Nyxol for Night Vision Disturbance (NVD). The study met the FDA-agreed upon primary endpoint with statistically significantly more Nyxol-treated subjects having gained 15 or more letters of mesopic low contrast distance visual acuity compared to placebo. Key secondary efficacy endpoints were also met with statistical significance, and Nyxol showed a favorable safety and tolerability profile with no serious adverse events reported. This is the first Phase 3 trial to study NVD and to show an improvement in distance and contrast vision at night with a pupil modulation mechanism which has cross-over safety differentiation to presbyopia. The Phase 3 protocol for VEGA-2 was submitted to the FDA in July.

- In June, the Company held a Type B Pre-NDA meeting with the FDA at which it received guidance on the content of CMC, clinical and non-clinical modules of the planned NDA for Nyxol in RM. The FDA confirmed that OraVerse and Regitine were appropriate to use as reference listed drugs to support a 505(b)(2) NDA.

Presentations, Publications, and Conferences

- From April through August 2022, Ocuphire was represented at conferences by Mina Sooch and several prominent key thought leaders, including David Boyer, MD, Peter Kaiser, MD, David Lally, MD, and Michael Allingham, MD, PhD who presented updates on APX3330 in DR, as well as Jay Pepose, MD, PhD, Marguerite McDonald, MD, Inder Paul Singh, MD, Ralph Chu, MD, James Katz, MD and Douglas Devries, OD who presented data for Nyxol in Presbyopia and RM. Since the beginning of the year, 9 podium papers, 5 posters, and over 10 panel or company talks were presented across a total of 20 medical, scientific, industry and investment conferences.

- In June at the 45th Annual Macula Society Meeting in Germany and in July at the American Society of Retina Specialists (ASRS) 2022 in New York, David Boyer, MD and David Lally MD, presented updated masked safety data from the ongoing ZETA-1 Phase 2b trial evaluating APX3330. The latest data from mid-June represented 100% of 103 subjects having completed 12 weeks or more and 70% having completed 24 weeks. These safety data are consistent with favorable safety profile from the prior 11 clinical trials with total exposure experience of over 9,000 subject-days with a 600 mg daily dose of APX3330.

- In July, at the 40th Annual Meeting of ASRS, David Boyer, MD, presented for the first time to the retina medical community a late-breaking paper highlighting positive data from the MIRA-2 and MIRA-3 Phase 3 registration trials for Nyxol eye drops to rapidly reverse mydriasis.

- In July, the Company announced the publication of a preclinical study supporting the novel mechanism of action inhibiting NFkB and the inflammatory pathway for APX3330 and APX pipeline candidates. The publication, titled “RelA is an Essential Target for Enhancing Cellular Responses to the DNA Repair/Ref-1 Redox Signaling Protein and Restoring Perturbated Cellular Redox Homeostasis in Mouse PDAC Cells” was published in *Frontiers in Oncology* and can be accessed here.

Corporate

- In April, Ocuphire appointed Jay Pepose, MD, PhD, as Chief Medical Advisor in addition to his Board of Directors role.

- Ocuphire was granted two new patents covering APX3330 and Nyxol:
    - APX3330 for Use in Diabetics: U.S. Patent No. 11,351,130 with claims to methods of treating inflammation and chronic pain in subjects suffering from diabetes using APX3330 was issued on June 7, 2022 with expiry in 2038.
    - Nyxol for Reversal of Mydriasis: U.S. Patent No. 11,400,077 with claims directed to methods for mydriasis treatment using phentolamine was issued on August 2, 2022 with expiry in 2039.

Second Quarter Ended June 30, 2022 Financial Highlights

As of June 30, 2022, Ocuphire had cash and cash equivalents of approximately $17.0 million. Based on current projections, management believes the current cash on hand will be sufficient to fund operations through the third quarter of 2023. Net cash used in operating

activities in the second quarter of 2022 was $3.8 million, with a cumulative total for the six months ended June 30, 2022 of $10.0 million.

General and administrative expenses for the three and six months ended June 30, 2022, were $1.8 million and $3.5 million, respectively, compared to $3.4 million and $5.million, respectively, for the three and six months ended June 30, 2021.

Research and development expenses for the three and six months ended June 30, 2022, were $3.2 million and $7.9 million, respectively, compared to $3.8 million and $7.3 million, respectively, for the three and six months ended June 30, 2021. The decrease from the comparable second quarter in 2021 was primarily attributable to the completion of clinical trials and the timing manufacturing activities for Nyxol and APX3330. The increase from the comparable six-month period in 2021 was primarily attributable to more ongoing clinical trials and manufacturing activities for Nyxol and APX3330 as well as regulatory, preclinical and other development activities.

The total loss from operations for the three and six months ended June 30, 2022, was $4.9 million and $11.4 million, respectively, compared to $7.1 million and $12.3 million, respectively, for the three and six months ended June 30, 2021.

Net loss for the three and six months ended June 30, 2022, was $4.9 million and $11.5 million, respectively, compared to $7.1 million and $46.2 million, respectively, for the three and six months ended June 30, 2021. Net loss per share for the three and six months ended June 30, 2022, was ($0.25) and ($0.60) per share, respectively, compared to ($0.52) and ($3.76) per share, respectively, for the comparable periods in 2021.

For further details on Ocuphire's financial results, refer to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 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 small-molecule therapies for the treatment of refractive and retinal eye disorders. 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 12 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 the VEGA-1 Phase 2 trial of Nyxol for treatment of presbyopia, both Nyxol as a single agent and Nyxol with low dose pilocarpine ("LDP") 0.4% as adjunctive therapy. The Company recently reported positive top-line results from LYNX-1 Phase 3 trial of Nyxol for NVD. 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 announced in March the completion of enrollment in the ZETA-1 Phase 2b clinical trial of APX3330 to treat

DR/DME. Please visit www.clinicaltrials.gov to learn more about Ocuphire's ongoing APX3330 Phase 2b trial in DR/DME ZETA-1 (NCT04692688) and completed Nyxol trials: Phase 3 registration trial in NVD LYNX-1 (NCT04638660), Phase 3 registration trials in RM MIRA-2 (NCT04620213) and MIRA-3 (NCT05134974), MIRA-4 Phase 3 pediatric safety study (NCT05223478), and Phase 2 trial in presbyopia VEGA-1 (NCT04675151). As part of its strategy, Ocuphire will continue to explore opportunities to acquire additional ophthalmic assets and 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 planned NDA filings, the results of the ZETA-1 Phase 2b trial, the results of the VEGA-2 Phase 3 trials, the market for Ocuphire's indications, business strategy, pre-commercialization activities, 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.

Contacts

Corporate

Mina Sooch, President & CEO
Ocuphire Pharma, Inc.
ir@ocuphire.com
www.ocuphire.com

Investors

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	
	June 30, 2022	**December 31, 2021**
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 17,025	$ 24,534
Prepaids and other current assets	740	1,314
Short-term investments	126	219
Total current assets	17,891	26,067
Property and equipment, net	8	10
Total assets	$ 17,899	$ 26,077
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,886	$ 1,584
Accrued expenses	1,418	1,733
Short-term loan	—	538
Total current liabilities	3,304	3,855
Warrant liabilities	—	—
Total liabilities	3,304	3,855
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of June 30, 2022 and December 31, 2021; no shares issued and outstanding at June 30, 2022 and December 31, 2021.	—	—

Common stock, par value $0.0001; 75,000,000 shares authorized as of June 30, 2022 and December 31, 2021; 20,099,602 and 18,845,828 shares issued and outstanding at June 30, 2022 and December 31, 2021, respectively.	2	2
Additional paid-in-capital	115,483	111,588
Accumulated deficit	(100,890)	(89,368)
Total stockholders' equity	14,595	22,222
Total liabilities and stockholders' equity	$ 17,899	$ 26,077

Ocuphire Pharma, Inc.
Condensed Consolidated 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,	
	2022	**2021**	**2022**	**2021**
Collaborations revenue	**$** —	$ 100	$ —	$ 100
Operating expenses:				
General and administrative	1,776	3,408	3,512	5,112
Research and development	3,162	3,829	7,934	7,311
Total operating expenses	4,938	7,237	11,446	12,423
Loss from operations	(4,938)	(7,137)	(11,446)	(12,323)
Interest expense	(4)	—	(9)	—
Fair value change of warrant liabilities	—	—	—	(33,829)
Other income (expense), net	15	1	(67)	2
Loss before income taxes	(4,927)	(7,136)	(11,522)	(46,150)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(4,927)	(7,136)	(11,522)	(46,150)
Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (4,927)	$ (7,136)	$ (11,522)	$ (46,150)
Net loss per share:				
Basic and diluted	$ (0.25)	$ (0.52)	$ (0.60)	$ (3.76)
Number of shares used in per share calculations:				

Basic and diluted	19,502,563	13,608,596	19,197,213	12,273,541

Source: Ocuphire Pharma