August 12, 2021

Ocuphire Announces Financial Results for the Second Quarter 2021 and Provides Corporate Update

Continued Momentum in Nyxol® Programs with Announcement of Positive Top-Line Results from VEGA-1 Phase 2 Trial in Presbyopia

Nyxol plus Low-Dose Pilocarpine Phase 2 Trial Results Show Potential for Best-in-Class Presbyopia Drug Profile in Data Presentations at 2021 ASCRS Meeting

$24M Cash Balance at Quarter-End Provides Runway Into Late 2022 Allowing Planned NDA Submission for Nyxol in Reversal of Mydriasis Indication

FARMINGTON HILLS, Mich., Aug. 12, 2021 (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 of 2021 and provided a corporate update.

“The second quarter of 2021 proved to be highly productive for Ocuphire, with continued success in clinical trials, key pivotal data presentations featured at major medical meetings across both Nyxol and APX3330, and additional IP protection granted for our lead drug candidate Nyxol,” said Mina Sooch, MBA, President and CEO of Ocuphire Pharma.

“Following our first quarter announcement of positive MIRA-2 Phase 3 results of Nyxol for the treatment of reversal of mydriasis (RM), we also announced positive top-line results from our VEGA-1 Phase 2 study, which evaluated Nyxol in combination with low-dose pilocarpine (LDP) for the treatment of presbyopia, the gradual loss of your eyes' ability to focus on nearby objects. In our view, RM and presbyopia both represent attractive and large US and global market opportunities, with each indication having a significant unmet medical need with very few if any pharmaceutical treatment options. Following the recent ASCRS meeting, it is also clear that global pharmaceutical and consumer healthcare companies are aggressively pursuing innovative new medicines, further validating the significant commercial potential for the large presbyopia market. Based upon the success of our VEGA-1 trial, we are highly confident that Nyxol in combination with LDP presents a potentially best-in-class therapeutic solution that can address the needs of the presbyopia patient population.”

“With our balance sheet recently strengthened in the second quarter combined with our capital efficient operations, Ocuphire is well positioned to deliver additional late-stage clinical milestones in 2022 for Nyxol and APX3330 while continuing our global business development efforts.”

Key Anticipated Future Milestones

- **Reversal of Mydriasis (RM):** Initiate second Phase 3 (MIRA-3) registration trial and a small pediatric trial (MIRA-4) in the second half of 2021 investigating Nyxol with results expected in early 2022; Planning to file NDA submission with FDA for Nyxol in RM indication in late 2022

- **Presbyopia:** Initiate Phase 3 program (VEGA-2/3) in first half of 2022 investigating Nyxol and LDP

- **Night Vision Disturbances:** Completion of enrollment expected by year-end 2021 and top-line data expected in early 2022 from Phase 3 (LYNX-1) registration trial investigating Nyxol

- **Diabetic Retinopathy and Diabetic Macular Edema:** Completion of enrollment in Phase 2 (ZETA-1) trial investigating APX3330 and top-line data expected in 2022

Second Quarter and Recent Business Highlights

Clinical Development

- In June, the Company announced successful results from the VEGA-1 Phase 2 trial of Nyxol plus low-dose pilocarpine (LDP) for the treatment of presbyopia; the trial met its primary endpoint of 3 lines of near vision improvement and multiple key secondary endpoints such as fast onset of action and durability with statistical significance and a favorable safety profile (including no headaches)

- In April, the Company initiated the ZETA-1 Phase 2 clinical trial to evaluate oral APX3330 in non-proliferative diabetic retinopathy (NPDR) and mild proliferative diabetic retinopathy (mild PDR)

Presentations and Publications

- In July, the Company announced publication in the Journal of Cellular Signaling featuring Ocuphire's novel oral Ref-1 inhibitor APX3330 in Phase 2 trial for the treatment of retinal disease which highlighted the favorable safety profile of APX3330 and its unique anti-angiogenic and anti-inflammatory mechanism of action properties relevant to a broad range of retinal diseases

- In July at the 2021 American Society of Cataract and Refractive Surgery (ASCRS) Annual Meeting, Dr. Jay S. Pepose, Medical Advisor and Board Director, presented two papers featuring the positive results Phase 2 Presbyopia (VEGA-1) and Phase 3 Reversal of Mydriasis (MIRA-2)

- In July, Mina Sooch, CEO, participated in the presbyopia drug therapy panel at the Eyecelerator held on July 22nd and in the Eye on Innovation panel at the Virtual Salon Series held on July 28th

- In late May, Ocuphire hosted a Key Opinion Leader Event on Nyxol as a potential new treatment option for reversing pharmacologically induced mydriasis, highlighting recent positive Phase 3 results from the MIRA-2 Phase 3 registrational study

- In May at the 2021 Association for Research in Vision and Ophthalmology (ARVO) Virtual Annual Meeting, Ocuphire presented data for APX3330 on pre-clinical ocular data and predictive human retina and plasma model data

Corporate

- Announced closing of a $15 million registered direct offering and just over $4 million from ATM, extending cash runway into late 2022

- Appointed Jay S. Pepose, M.D., Ph.D. to the Company's Board of Directors

- Received two new U.S. patent grants covering Nyxol® including Nyxol plus LDP claims for the treatment of presbyopia through 2039

- Entered into a license agreement granting Processa Pharmaceuticals (Nasdaq: PCSA) an exclusive license to develop, manufacture and commercialize globally RX-3117 (Rexahn legacy oncology intellectual property), excluding China, Hong Kong, Macau, Republic of Singapore and Taiwan (already licensed to BioSense Global LLC ("BioSense"))

- Ocuphire (Nasdaq: OCUP) added to the Russell Microcap® Index

Second Quarter and Year to Date 2021 Financial Highlights

As of June 30, 2021, the Company had cash and cash equivalents of approximately $24.2 million. Net cash used in operating activities for the three and six months ended June 30, 2021, was $4.3 million and $10.1 million, respectively.

Collaborations revenue was $0.1 million for the three and six months ended June 30, 2021. Revenue during the periods was derived from the license agreement with BioSense related to certain technology transfers. There was no collaborations revenue recognized during the comparable prior year periods.

General and administrative expenses for the three and six months ended June 30, 2021, were $3.4 million and $5.1 million, respectively, compared to $0.6 million and $0.9 million, respectively, for the three and six months ended June 30, 2020. The increases from the comparable periods in 2020 were attributable to increased costs primarily in administrative employee headcount, stock-based compensation, insurance, legal and settlement costs, and costs associated with operating as a public company subsequent to the reverse merger.

Research and development expenses for the three and six months ended June 30, 2021, were $3.8 million and $7.3 million, respectively, compared to $0.7 million and $0.9 million, respectively, for the three and six months ended June 30, 2020. The increases from the

comparable periods in 2020 were primarily attributable to four new clinical trials and manufacturing activities for Nyxol and APX3330 as well as regulatory, preclinical, and other development activities.

There were no acquired in-process research and development expenses in the current six-month period. In the prior year in connection with the sublicense agreement with Apexian for the continued research, development and potential commercialization of APX3330, the Company recorded acquired in-process research and development expenses of $2.1 million during the six-month period ended June 30, 2020.

The total loss from operations for the three and six months ended June 30, 2021 was $7.1 million and $12.3 million, respectively, compared to $1.3 million and $4.0 million for the three and six months ended June 30, 2020, respectively. This included non-cash stock-based compensation expense of $0.5 million and $1.0 million during the three and six months ended June 30, 2021, respectively, and $0.3 million and $0.4 million during the three and six months ended June 30, 2020, respectively.

For further details on Ocuphire's financial results, including results for the three and six month periods ended June 30, 2021 refer to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, 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 front and back of the eye indications. The company's lead product candidate, Nyxol® (0.75% phentolamine ophthalmic solution) Eye Drops, 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 dim light or night vision disturbances (NVD), reversal of pharmacologically-induced mydriasis (RM), and presbyopia, and has been studied in 9 clinical trials including the recently completed Phase 3 trial in RM and Phase 2 trial in presbyopia. Ocuphire reported positive topline data in March 2021 for MIRA-2, a Phase 3 FDA registration study for treatment of RM. Ocuphire also reported positive top-line data in June 2021 for VEGA-1, a Phase 2 trial for the treatment of presbyopia. Nyxol is also currently in Phase 3 clinical development 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. APX3330 is currently enrolling subjects in a Phase 2 clinical trial in subjects with DR/DME. 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. Please visit www.clinicaltrials.gov to learn more about Ocuphire's completed Phase 2 trials, recently completed Phase 3 registration trial in RM (NCT04620213), recently completed Phase 2 trial in presbyopia (NCT04675151), ongoing Phase 3 registration trial in NVD (NCT04638660), and Phase 2 trial in DR/DME (NCT04692688). For more information, please 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 Nyxol plus LDP's potential to be a 'best-in-class' presbyopia treatment option, the US and global market and commercial potential of Nyxol alone or in combination with LDP, the expected timing of our future clinical trials in RM, NVD, presbyopia, and DR/DME, and the extent of the Company's cash runway. 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.

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

	As of	
	Jun 30,	**Dec 31,**

	2021	2020
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 24,234	$ 16,399
Collaborations receivable	50	—
Prepaids and other assets	956	1,269
Total current assets	25,240	17,668
Property and equipment, net	12	14
Total assets	$ 25,252	$ 17,682
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 1,496	$ 1,214
Accrued expenses	1,203	1,971
Total current liabilities	2,699	3,185
Warrant liabilities	—	27,964
Total liabilities	2,699	31,149
Commitments and contingencies		
Stockholders' equity (deficit):		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of June 30, 2021 and December 31, 2020; no shares issued and outstanding at June 30, 2021 and December 31, 2020.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of June 30, 2021 and December 31, 2020; 16,891,855 and 10,882,495 shares issued and outstanding at June 30, 2021 and December 31, 2020, respectively.	2	1
Additional paid-in-capital	101,376	19,207
Accumulated deficit	(78,825)	(32,675)
Total stockholders' equity (deficit)	22,553	(13,467)
Total liabilities and stockholders' equity	$ 25,252	$ 17,682

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,	
	2021	2020	2021	2020
Collaborations revenue	$ 100	$ —	$ 100	$ —
Operating expenses:				
General and administrative	3,408	551	5,112	942
Research and development	3,829	711	7,311	929
Acquired in-process research and development	—	—	—	2,126
Total operating expenses	7,237	1,262	12,423	3,997
Loss from operations	(7,137)	(1,262)	(12,323)	(3,997)
Interest expense	—	(689)	—	(1,243)
Fair value change of warrant liability and premium conversion derivatives	—	(919)	(33,829	(721)
Gain on note extinguishment	—	1,260	—	1,260
Other income	1	6	2	9
Loss before income taxes	(7,136)	(1,604)	(46,150)	(4,692)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(7,136)	(1,604)	(46,150)	(4,692)
Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (7,136)	$ (1,604)	$ (46,150)	$ (4,692)
Net loss per share:				
Basic and diluted	$ (0.52)	$ (0.43)	$ (3.76)	$ (1.29)
Number of shares used in per share calculations:				
Basic and diluted	13,608,596	3,743,907	12,273,541	3,645,948

Source: Ocuphire Pharma