November 12, 2021

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

On Track to Initiate Additional Phase 3 FDA Registration Trials for Nyxol® Eye Drops in Reversal of Mydriasis (RM) in 4Q21 and Presbyopia in 1H22

Three Clinical Trial Data Readouts Expected in Early 2022 for Nyxol in Night Vision Disturbance, RM, and RM for Pediatric Patients

Planned NDA Submission for Nyxol in Reversal of Mydriasis Indication in Late 2022

More Publications Supporting Novel Transcription Factor (Ref-1) Inhibitor, APX3330, Targeting Both Neovascularization and Inflammation in Retinal Diseases

Currently Recruiting for Phase 2 Trial Evaluating APX3330 for the Treatment of Diabetic Retinopathy with Data Expected in 2H22

FARMINGTON HILLS, Mich., Nov. 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 third quarter of 2021 and provided a corporate update.

“The third quarter marked continued progress across our late-stage clinical programs and opportunities for multiple data presentations at major medical meetings,” said Mina Sooch, MBA, President and CEO of Ocuphire Pharma. “We have already achieved two successful clinical trials for Nyxol. In reversal of mydriasis (RM), we reported positive results in a Phase 3 trial and are on track to initiate the second Phase 3 trial before year end. In presbyopia, we reported positive results in a Phase 2 clinical trial. We are also delighted to see the early US regulatory approval of Allergan’s VUITY™ eye drops, the first pharmaceutical therapy for the large presbyopia market.”

“We are also very pleased to see a growing body of supportive research for our Phase 2 oral drug candidate, APX3330, which inhibits known pro-angiogenic and pro-inflammatory pathways. As a highly differentiated, first-in-class and orally-delivered therapy, we believe APX3330 will be an important source of potential value creation with the opportunity to broadly address the unmet global clinical need in diabetic retinopathy and treatment burden in other retinal diseases.”

“This week marks Ocuphire’s one-year anniversary of public trading on the Nasdaq and we are proud to have achieved so many important clinical and business milestones in that time. We thank our clinical trial participants and investigators for their continued support. Looking

ahead, we believe 2022 is shaping up to be an even more exciting and catalyst-rich year to build significant value for our company and our shareholders, with cash on hand that provides runway into late 2022 to achieve these milestones."

Key Anticipated Future Milestones

- **Reversal of Mydriasis (RM):** Initiate second Phase 3 (MIRA-3) registration trial in subjects 12 and older and a small pediatric trial in subjects ages 3 to 11 (MIRA-4) in the fourth quarter 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) in first half of 2022 investigating Nyxol and Low-Dose Pilocarpine (LDP)

- **Night Vision Disturbances (NVD):** Top-line data expected in early 2022 from Phase 3 (LYNX-1) registration trial investigating Nyxol

- **Diabetic Retinopathy (DR) and Diabetic Macular Edema (DME):**Top-line data expected in the second half of 2022 for the randomized, well-controlled Phase 2 (ZETA-1) trial investigating APX3330

Third Quarter and Recent Business Highlights

Presentations and Publications

- In November, clinical data on Nyxol® and APX3330 were accepted for presentation at poster sessions at the American Academy of Ophthalmology (AAO) 2021 annual meeting to take place in New Orleans, November 12 – 15. In addition, Ocuphire presented new data on improvement in intermediate vision and Snellen equivalent near vision at the Eyecelerator@AAO 2021 conference on November 11. Ocuphire was one of two companies presenting clinical data for presbyopia at this meeting.

- In October, the Company announced the publication of a review article within the Special Issue "Advances in Molecular Activity of Potential Drugs" of the *International Journal of Molecular Sciences,* focused on how novel inhibitors of APE1/Ref-1 such as APX3330 may have the potential to improve disease outcomes for retinal disease patients**.** The article underscores the role of the APE1/Ref-1 protein in pro-angiogenic pathways associated with neovascular eye disease including diabetic retinal diseases and age-related macular degeneration. It can be accessed online at the following link: Inhibition of APE1/Ref-1 for Neovascular Eye Disease: From Biology to Therapy.

- In October, the Company announced the publication of a review article in *Cells* titled "Potential Therapeutic Candidates for Age-Related Macular Degeneration" noting the potential of APX3330 (referred to as "E3330") for the treatment of age-related macular degeneration (AMD). Because APE1/Ref-1 has been shown to contribute to retinal angiogenesis, the authors conclude that APE1/Ref-1 inhibitors such as APX3330 could inhibit the abnormal blood vessel formation seen in AMD by reducing retinal endothelial cell proliferation, migration, and tube formation. The article can be accessed online at the following link: Potential Therapeutic Candidates for Age-Related Macular Degeneration (AMD).

- In October, Michael J. Allingham, MD, PhD presented at the39th Annual Scientific Meeting of the American Society of Retina Specialists (ASRS) (Diabetic Retinopathy 1 Symposium), highlighting the favorable safety and tolerability data for APX3330 in over 300 healthy volunteers and cancer/inflammation disease patients across 11 Phase 1 and Phase 2 studies. Also, Mina Sooch, CEO, presented APX3330 history and the design of the ongoing Phase 2 trial in DR at the OIS Retina Innovation Summit@ASRS.

- In July, the Company announced publication in theJournal 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 papers featuring positive results for Nyxol in two studies: Phase 2 Presbyopia (VEGA-1) and Phase 3 Reversal of Mydriasis (MIRA-2). The Phase 3 MIRA-2 data presentation at ASCRS won the Best Paper of the Session.

- In July, Mina Sooch, CEO, participated in the presbyopia drug therapy panel at the Eyecelerator@ASCRS 2021 held on July 22nd and in the Eye on Innovation panel at the Virtual Salon Series held on July 28th.

Intellectual Property

- U.S. Patent and Trademark Office issued patent no. 11,160,770 "Compounds, compositions and methods for treating oxidative DNA damage disorders" which provides protection for APX2009 and other APX pipeline candidates.

Third Quarter and Year-To-Date 2021 Financial Highlights

As of September 30, 2021, the Company had cash and cash equivalents of approximately $22.2 million. Net cash used in operating activities for the nine months ended September 30, 2021 was $13.7 million.

Collaborations revenue was $0.5 million and $0.6 million for the three months and nine months ended September 30, 2021, respectively. Revenue during the periods was derived from the license agreements with Biosense Global, LLC and Processa Pharmaceuticals, Inc. related to certain technology transfers. There was no collaborations revenue recognized during the comparable prior year periods.

General and administrative expenses for the three months and nine months ended September 30, 2021 were $1.6 million and $6.7 million, respectively, compared to $0.6 million and $1.5 million for the comparable periods in 2020, respectively. The increases in the current periods were primarily attributable to administrative employee headcount, stock-based compensation, professional services, 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 months and nine months ended

September 30, 2021 were $3.1 million and $10.4 million, respectively, compared to $1.4 million and $2.3 million for the comparable periods in 2020, respectively. In the current periods, the increases were primarily attributable to new clinical trials and manufacturing activities for Nyxol and APX3330 as well as regulatory, preclinical and other development activities.

The loss from operations for the three and nine months ended September 30, 2021 was $4.2 million and $16.6 million, respectively, compared to $1.9 million and $5.9 million for the three and nine months ended September 30, 2020, respectively.

There was a non-cash expense of $33.8 million related to fair value change in warrant liabilities recorded for the nine months ended September 30, 2021 compared to a benefit of $0.2 million recorded for the nine months ended September 30, 2020 related to premium conversion derivatives. The reported losses also included non-cash stock-based compensation expense of $0.5 million and $1.4 million during the three and nine months ended September 30, 2021, respectively, and $0.6 million and $1.0 million during the three and nine months ended September 30, 2020, respectively.

For further details on Ocuphire's financial results refer to the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, as 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 reversal of pharmacologically-induced mydriasis (RM), presbyopia and dim light or night vision disturbances (NVD), 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 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.

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 Consolidated Balance Sheets
(in thousands, except share amounts and par value)

	As of	
	September 30, 2021	December 31, 2020
	(unaudited)	

Assets

Current assets:		
Cash and cash equivalents	$ 22,250	$ 16,399
Short-term investments	383	—
Prepaids and other assets	560	1,269
Total current assets	23,193	17,668
Property and equipment, net	11	14
Total assets	$ 23,204	$ 17,682
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 1,434	$ 1,214
Accrued expenses	1,204	1,971
Total current liabilities	2,638	3,185
Warrant liabilities	—	27,964
Total liabilities	2,638	31,149
Commitments and contingencies		
Stockholders' equity (deficit)		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of September 30, 2021 and December 31, 2020; no shares issued and outstanding at September 30, 2021 and December 31, 2020.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of September 30, 2021 and December 31, 2020; 17,295,434 and 10,882,495 shares issued and outstanding at September 30, 2021 and December 31, 2020, respectively.	2	1
Additional paid-in-capital	103,619	19,207
Accumulated deficit	(83,055)	(32,675)
Total stockholders' equity (deficit)	20,566	(13,467)
Total liabilities and stockholders' equity (deficit)	$ 23,204	$ 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 September 30,		For the Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Collaborations revenue	**$** 489	$ —	$ 589	$ —

Operating expenses:				
General and administrative	1,595	565	6,707	1,508
Research and development	3,126	1,383	10,437	2,311
Acquired in-process research and development	—	—	—	2,126
Total operating expenses	4,721	1,948	17,144	5,945
Loss from operations	(4,232)	(1,948)	(16,555)	(5,945)
Interest expense	—	(179)	—	(1,422)
Fair value change of warrant liability and premium conversion derivatives	—	879	(33,829)	158
Gain on note extinguishment	—	—	—	1,260
Other income, net	2	—	4	9
Loss before income taxes	(4,230)	(1,248)	(50,380)	(5,940)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(4,230)	(1,248)	(50,380)	(5,940)
Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (4,230)	$ (1,248)	$ (50,380)	$ (5,940)
Net loss per share:				
Basic and diluted	$ (0.25)	$ (0.33)	$ (3.64)	$ (1.61)
Number of shares used in per share calculations:				
Basic and diluted	16,925,006	3,743,907	13,841,067	3,678,840

Source: Ocuphire Pharma