May 7, 2021

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

Reported Positive Data in First Pivotal Phase 3 Trial for Nyxol Eye Drops with Plans to Advance Towards NDA Submission and Commercialization in Reversal of Mydriasis Indication

Demonstrated Execution Capability with Four Phase 3 and 2 Clinical Trials Initiated and/or Completed in 1Q21

Second Candidate APX3330 Actively Enrolling Diabetic Retinopathy Patients has the Potential to be a Novel Oral Treatment Option with Anti-VEGF and Anti-Inflammatory Mechanisms

FARMINGTON HILLS, Mich., May 07, 2021 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of several eye disorders, today announced financial results for the first quarter of 2021 and provided a corporate update.

“The highlight of the first quarter was our announcement of positive top-line results from the pivotal MIRA-2 Phase 3 trial investigating Nyxol® eye drops for reversal of pharmacologically-induced mydriasis (pupil dilation). The highly statistically significant results provide the foundation to advance Nyxol towards NDA submission in an indication representing over 100 million annual eye exams with no commercial treatments currently available”, said Mina Sooch, MBA, President and CEO of Ocuphire Pharma. “The recent Phase 3 data further validate Nyxol’s unique mechanism of action on the iris dilator muscle, its consistent therapeutic effect of pupil diameter reduction, and its favorable safety and tolerability profile for additional refractive indications including Presbyopia and Night Vision Disturbances. We are also making progress with our second product candidate, APX3330, and are currently enrolling patients in a Phase 2 trial primarily in Diabetic Retinopathy. Overall, 2021 is off to a strong start and we look forward to more updates on our programs throughout the year including the Phase 2 Presbyopia data readout expected at the end of June.”

Cam Gallagher, MBA, Chair of Ocuphire’s Board of Directors commented, “Since our public listing via reverse merger just six months ago, the management team together with Ocuphire’s clinical and manufacturing partners have successfully launched an impressive four Phase 3 and 2 clinical trials for Nyxol and APX3330, clearly demonstrating their execution capabilities. Importantly, data from multiple trials at Ocuphire have been published in several peer-reviewed journals and presented at multiple ophthalmic and healthcare

banking conferences, further establishing our presence within the industry. We look forward to continuing this momentum in 2021, and with our recent positive Phase 3 data, we are excited to move ahead with our commercialization strategies for Nyxol."

Key Anticipated Future Milestones

- **Presbyopia:** Top-line data expected end of Q2 2021 for Phase 2 VEGA-1 trial investigating a kit combination of Nyxol and low-dose 0.4% pilocarpine
- **Reversal of Mydriasis:** Presenting Phase 3 MIRA-2 results at the 2021 American Society of Cataract and Refractive Surgeons conference in Las Vegas in July
- **Night Vision Disturbances:** Top-line data expected end of Q3 2021 for pivotal Phase 3 LYNX-1 trial investigating Nyxol
- **Reversal of Mydriasis:** Planning to initiate second Phase 3 MIRA-3 registration trial in 2H 2021 investigating Nyxol with results expected in early 2022
- **Diabetic Retinopathy and Diabetic Macular Edema:** Completion of enrollment in Phase 2 ZETA-1 trial investigating APX3330 with top-line data expected early 2022

First Quarter and Recent Business Highlights

Clinical Development

- Reported positive results in pivotal Phase 3 MIRA-2 trial investigating Nyxol for Reversal of Mydriasis (within 4 months of initiation) which met primary and multiple secondary endpoints demonstrating a more rapid return to baseline pupil diameter after dilation across multiple commonly used dilating agents and iris colors
- Initiated ZETA-1 Phase 2 trial investigating the first-in-class oral anti-VEGF and anti-inflammatory APX3330 in Diabetic Retinopathy and Diabetic Macular Edema
- Initiated Phase 2 VEGA-1 trial evaluating a kit combination of Nyxol and low-dose pilocarpine in Presbyopia with a differentiated pharmacologic approach that moderately acts on both the iris dilator and iris sphincter muscles that control pupil diameter

Presentations and Publications

- Presented positive pre-clinical data supporting oral APX3330's efficacy and sufficient exposure to the retina at the Association for Research in Vision and Ophthalmology (ARVO) virtual Annual Meeting
- Published positive results from the MIRA-1 Phase 2b clinical trial evaluating the safety and efficacy of Nyxol for Reversal of Mydriasis in *Optometry and Visual Science* journal
- Presented highlights of Nyxol presbyopia program at the 2021 Ophthalmology Innovation Summit (OIS) Presbyopia Innovation Showcase

First Quarter 2021 Financial Highlights

As of March 31, 2021, the Company had cash and cash equivalents of approximately $10.6 million.

General and administrative expenses for the three months ended March 31, 2021 were $1.7 million compared to $0.4 million for the three months ended March 31, 2020. The $1.3 million increase was primarily attributable to an increase in administrative employee headcount, stock-based compensation, professional services, insurance, and legal costs associated with the operating as a public company in the current period subsequent to the merger.

Research and development expenses for the three months ended March 31, 2021 were $3.5 million compared to $0.2 million for the three months ended March 31, 2020. The $3.3 million increase as compared to the prior period was primarily attributable to four new clinical trials and manufacturing activities for Nyxol and APX3330 as well as regulatory, preclinical, and other development activities.

The total GAAP loss from operations for the three months ended March 31, 2021 was $5.2 million compared to $2.7 million for the three months ended March 31, 2020. This included non-cash stock-based compensation expense of $494,000 and $61,000, respectively.

The quarter ended March 31, 2021 included a non-cash charge of $33.8 million related to the fair value change in warrant liabilities included in other expense. The quarter ended March 31, 2020 included a non-cash charge of $0.6 million related to interest expense on convertible notes and a $0.2 million non-cash benefit due to the fair value change in premium conversion derivatives related to convertible notes, both included in other expense. As a result, GAAP net loss attributable to common stockholders for the quarter ended March 31, 2021 was $39.0 million compared to $3.1 million for the quarter ended March 31, 2020.

Effective February 3, 2021, each investor that invested in the Pre-Merger Financing entered into a Waiver Agreement with Ocuphire. The Waiver Agreements provide for the elimination of the full ratchet anti-dilution provisions contained in the Series A Warrants (as certain of the anti-dilution provisions had previously caused liability accounting treatment for the Series A Warrants). Upon the effective date of the Waiver Agreements, the Series A Warrants were reclassified to equity. In addition, the investors agreed to waive certain rights, finalize the exercise price and number of Series A Warrants and Series B Warrants, eliminate certain financing restrictions, extend the term of certain leak-out agreements, and, in the case of certain investors, grant certain registration rights for the shares underlying the Series A Warrants.

Non-GAAP adjusted net loss was $5.2 million or ($0.47) per share for the three months ended March 31, 2021, compared with a non-GAAP adjusted net loss of $3.3 million or ($0.93) per share for the three months ended March 31, 2020. Non-GAAP adjusted net loss for the three months ended March 31, 2021 and 2020 excludes expenses for the fair value change in warrant liabilities related to the Pre-Merger Financing and premium conversion derivatives related to convertible notes, respectively. See "Non-GAAP Financial Measures" and "Reconciliation of GAAP to Non-GAAP Financial Measures" below for a reconciliation of this GAAP and non-GAAP financial measure.

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, 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 – Nyxol and APX3330 – targeting front and back of the eye indications. 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 in key global markets. For more information, please visit www.ocuphire.com.

About Nyxol

Ocuphire'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. Nyxol has been studied in 8 clinical trials demonstrating a favorable safety and tolerability profile. Ocuphire recently reported positive top-line data for pivotal MIRA-2 Phase 3 trial for treatment of RM. Nyxol is also currently in Phase 2 for presbyopia, with top-line results expected Q2 2021, and in Phase 3 clinical development for NVD with top-line results expected Q3 2021. Please visit www.clinicaltrials.gov to learn more about Ocuphire's completed Phase 2 trials in RM, Glaucoma, and NVD, recently completed Phase 3 registration trial in RM (NCT04620213), and ongoing Phase 2 trial in presbyopia (NCT04675151) and Phase 3 registration trial in NVD (NCT04638660).

About APX3330

Ocuphire's second product candidate, APX3330, is a small molecule oral drug candidate and a first-in-class inhibitor of the transcription factor regulator Ref-1 (reduction-oxidation effector factor-1). With its novel mechanism of action, APX3330 blocks the downstream pathways regulated by Ref-1, specifically decreasing abnormal activation of both angiogenesis (VEGF) and inflammatory (NF-kB) pathways that are relevant to retinal and choroidal vascular diseases, including diabetic retinopathy (DR), diabetic macular edema (DME), and age-related macular degeneration (AMD). APX3330 has been studied in 11 Phase 1 and 2 trials and has demonstrated a favorable safety and tolerability profile in over 300 oncology and hepatic patients. APX3330 is actively recruiting in a Phase 2 trial in DR/DME, with results expected in early 2022. Please visit www.clinicaltrials.gov for more information about Ocuphire's ongoing Phase 2 trial in DR/DME (NCT04692688).

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

Ocuphire's product candidates, results of ongoing and future clinical trials, and commercialization and market opportunities. 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, and (ix) the success and timing of commercialization of any of Ocuphire's product candidates. 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.

Note: All educational content of the ASCRS ASOA Annual Meeting is planned by its program committee, and ASCRS ASOA does not endorse, promote, approve, or recommend the use of any products, devices, or services.

Ocuphire Contacts

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

Corey Davis, Ph.D.
LifeSci Advisors
cdavis@lifescieadvisors.com

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

	As of	
	March 31, 2021	**December 31, 2020**
	(unaudited)	
Assets		
Current assets:		

Cash and cash equivalents	$ 10,597	$ 16,399
Prepaids and other assets	1,428	1,269
Deferred costs	88	—
Total current assets	12,113	17,668
Property and equipment, net	13	14
Total assets	$ 12,126	$ 17,682
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable	$ 1,415	$ 1,214
Accrued expenses	895	1,971
Total current liabilities	2,310	3,185
Warrant liabilities	—	27,964
Total liabilities	2,310	31,149
Commitments and contingencies		
Stockholders' equity (deficit)		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of March 31, 2021 and December 31, 2020; no shares issued and outstanding at March 31, 2021 and December 31, 2020.	—	—
Common stock, par value $0.0001; 75,000,000 shares authorized as of March 31, 2021 and December 31, 2020; 10,929,881 and 10,882,495 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively.	1	1
Additional paid-in-capital	81,504	19,207
Accumulated deficit	(71,689)	(32,675)
Total stockholders' equity (deficit)	9,816	(13,467)
Total liabilities and stockholders' equity (deficit)	$ 12,126	$ 17,682

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

	Three Months Ended March 31,	
	2021	**2020**
Operating expenses:		
General and administrative	$ 1,704	$ 391
Research and development	3,482	218
Acquired in process research and development	—	2,126

Total operating expenses	5,186	2,735
Loss from operations	(5,186)	(2,735)
Interest expense	—	(554)
Fair value change in warrant liabilities and premium conversion derivatives	(33,829)	198
Other income	1	3
Loss before income taxes	(39,014)	(3,088)
Benefit (provision) for income taxes	—	—
Net loss	(39,014)	(3,088)
Other comprehensive loss, net of tax	—	—
Comprehensive loss	$ (39,014)	$ (3,088)
Net loss per share:		
Basic and diluted	$ (3.57)	$ (0.87)
Number of shares used in per share calculations:		
Basic and diluted	10,923,651	3,547,990

Non-GAAP Financial Measures

In addition to operating results as calculated in accordance with Generally Accepted Accounting Principles (GAAP), Ocuphire uses certain non-GAAP financial measures when evaluating operational performance. The following table presents the Company's net loss and net loss per common share calculated in accordance with GAAP and as adjusted to remove the impact of certain non-cash charges. Ocuphire's management believes that these non-GAAP financial measures are useful to enhance understanding of the Company's financial performance and are more indicative of its operational performance and facilitate a better comparison among fiscal periods.

These non-GAAP financial measures are not, and should not be viewed as, substitutes for GAAP reporting measures. These non-GAAP financial measures are not based on any comprehensive set of accounting rules or principles, differ from GAAP measures with the same names, and may differ from non-GAAP financial measures with the same or similar names that are used by other companies. Ocuphire believes that non-GAAP financial measures should only be used to evaluate its results of operations in conjunction with the corresponding GAAP financial measures. Ocuphire encourages investors to carefully consider its results under GAAP, as well as the reconciliations between these presentations, to more fully understand our business.

Non-GAAP adjusted net loss and non-GAAP adjusted net loss per share exclude the fair value change in warrant liabilities and premium conversion derivatives. Ocuphire excludes these items because they are non-cash expenses and have no direct correlation to the operation of its business.

Ocuphire Pharma, Inc.
Reconciliation of GAAP to Non-GAAP Financial Measures
(in thousands, except share and per share amounts)
(unaudited)

A reconciliation between GAAP Net Loss to Non-GAAP adjusted Net Loss and GAAP Net

Loss per common share to Non-GAAP adjusted Net Loss per common share:

	Three Months Ended March 31,	
	2021	**2020**
GAAP Net Loss	$ (39,014)	$ (3,088)
Adjustments:		
Fair value change in warrant liabilities and premium conversion derivatives[(1)]	33,829	(198)
Non-GAAP adjusted Net Loss	$ (5,185)	$ (3,286)
GAAP Net Loss per common share, Basic and diluted	$ (3.57)	$ (0.87)
Adjustment to Net Loss per common share	3.10	(0.06)
Non-GAAP adjusted Net Loss per common share, Basic and diluted	$ (0.47)	$ (0.93)
Number of shares used in per share calculations:		
Basic and diluted	10,923,651	3,547,990

1. To reflect a non-cash charge to other expense for the fair value change in warrant liabilities and premium conversion derivatives. The $33.8 million fair value change in warrant liabilities was due primarily to the issuance of the Series A warrants in connection with the Pre-Merger Financing and to the fluctuations in Ocuphire's common stock fair value (between December 31, 2020 and February 3, 2021) and other factors.

SOURCE: Ocuphire Pharma, Inc.

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