March 24, 2022

Ocuphire Announces Financial Results for the Fourth Quarter and Year Ended December 31, 2021 and Provides Corporate Update

Building Momentum with Completion of Patient Enrollment in 4 Late-Stage Trials Multiple Late-Stage Clinical Trial Data Catalysts in 2022:
– Nyxol® MIRA-3 Phase 3 Results Expected End of 1Q 2022
– MIRA-4 Pediatric, and LYNX-1 Phase 3 Results Expected to follow in2Q 2022
– ZETA-1 Phase 2 Diabetic Retinopathy Results Expected in 2H 2022

NDA Filing for Nyxol in Reversal of Mydriasis Planned for Late 2022 with Potential Launch as First Dilation Reversal Drop in 2H 2023

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

FARMINGTON HILLS, Mich., March 24, 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 fourth quarter and year ended December 31, 2021 and provided a corporate update.

“2021 proved to be a highly productive year for Ocuphire with 2022 setting up to be a more transformative year given our series of late-stage data read-outs in MIRA, LYNX and ZETA trials throughout the year, ending with our first planned NDA filing. We have an ambitious vision in ophthalmology targeting highly prevalent refractive and diabetic retinal diseases with our 2 lead small molecule drug candidates,” said Mina Sooch, MBA, Founder and CEO of Ocuphire Pharma. “We are pleased to rapidly exceed enrollment in and complete 4 clinical trials across Nyxol and APX3330 in the first months of 2022. At our R&D Day in January, we reported for the first time positive Phase 2 results in presbyopia for Nyxol as a single-agent. With this new chronic opportunity for Nyxol alone as a pupil modulation agent, we can potentially realize synergies in presbyopia and NVD patients. We recently held a FDA Type-C meeting and gained clear guidance for the VEGA Phase 3 presbyopia program, for which we plan to initiate in mid-2022. With the successful enrollment of the 24-week study for our retinal candidate APX3330 and the continued favorable systemic and ocular safety profile that we shared at our recent R&D Day, we are also excited to lead the retinal landscape with an oral option for diabetic retinopathy patients and report our topline data from our placebo-controlled, double-masked, Phase 2b ZETA-1 trial in the second half of 2022.”

Jay Pepose, MD, PhD, Ocuphire's Medical Advisor and Board Member stated, "Ocuphire's product candidates, if approved, would give eye care practitioners the ability to enhance their patients' vision and overall experience. As a refractive surgeon, I am particularly excited about Nyxol for RM because there is currently no FDA approved commercially available product to treat this major clinical need and patient complaint. For presbyopia, I am impressed by Nyxol's favorable tolerability profile and durable near vision improvements for at least 12 hours across a broad age of patients (40 to 64 years old) in the recent VEGA-1 Phase 2 trial. Nyxol is differentiated from the other miotics in the presbyopia landscape by its mechanism of action inhibiting the iris dilator muscle to achieve an optimal pupil size. Since Nyxol does not engage the iris sphincter or ciliary muscle, as a single drop, this may become a viable treatment option for presbyopia patients with high myopia, for whom miotics are contraindicated because of the risk of retinal detachment."

Cam Gallagher, Chairman of the Board for Ocuphire added, "In the past year, Ocuphire has elevated its profile within the ophthalmology and optometry medical community and we are delighted to have expanded our prestigious Medical Advisory Board to over 15 refractive and retinal KOLs. The team led by Mina has executed and delivered on several key clinical development milestones and set the momentum for a catalyst-rich 2022 that has the potential to build significant value for our company and shareholders."

Key Anticipated Future Milestones

- **Reversal of Mydriasis (RM):**
    - **MIRA-3:** Report topline results from the Phase 3 MIRA-3 registration trial at the end of 1Q 2022
    - **MIRA-4:** Report topline results from pediatric safety trial in 2Q 2022
    - **New Drug Application (NDA):** If the results are positive from the ongoing MIRA trials, expect to file an 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% LDP as adjunctive treatment; and, if successful, expect to file an NDA in 2023
- **Night Vision Disturbances (NVD):** Report top-line results in 2Q 2022 from the Nyxol Phase 3 LYNX-1 trial
- **Diabetic Retinopathy (DR) and Diabetic Macular Edema (DME):**Report top-line results from the APX3330 Phase 2b ZETA-1 trial in 2H 2022

Fourth Quarter and Recent Business Highlights

Corporate

- In January 2022, the Company held an Investor R&D Day webinar that featured six ophthalmic Key Opinion Leaders (KOLs): 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 from retina practice areas who discussed the unmet

needs in RM, presbyopia and DR being 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 December 2021, the Company strengthened its Medical Advisory Board with the addition of six world-class KOLs: David Brown, MD, FACS; David Lally, MD; Y. Ralph Chu, MD; James Katz, MD; Mitchell Jackson, MD; and Douglas Devries, OD.

Clinical Development

- In March 2022, the Company completed enrollment of 103 (target of 80-100) diabetic retinopathy patients in the ZETA-1 Phase 2b trial of first-in-class oral APX3330. Masked safety data from the trial, announced during the R&D Day event in January 2022, demonstrated a favorable safety profile, consistent with prior studies with additional exposure data in diabetic patients with retinal disease.

- In March 2022, the Company completed enrollment in MIRA-4 Trial for Nyxol in RM by enrolling 23 healthy (target of 20) pediatric subjects ages 3-11 years.

- In February 2022, the Company completed enrollment in MIRA-3 Pivotal Phase 3 Trial for Nyxol in RM, surpassing its enrollment target of 330 with 368 patients ages 12 years and over.

- In February 2022, Ocuphire held a Type-C meeting with the FDA from which it obtained guidance regarding the design of pivotal studies and clarification of the CMC and other data requirements for filing an NDA to seek approvals of Nyxol for the treatment of presbyopia, both as a single agent and with LDP as adjunct eye drops. This represents our third Type-C or Type-B End of Phase 2 meeting with FDA for the Nyxol program across indications.

- In January 2022, the Company completed enrollment of LYNX-1 Phase 3 Trial investigating Nyxol for the treatment of night vision disturbances (NVD) in 145 patients (target of 140).

- In January 2022, 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 efficacy and long durability compared to placebo at 12 hours post-dosing. The Company plans to proceed with the Phase 3 VEGA program to potentially support 2 NDAs: Nyxol as a single drop and Nyxol with low-dose pilocarpine (LDP) as adjunctive treatment.

Presentations, Publications, and Conferences

- In February 2022, David Boyer, MD, presented at the Angiogenesis, Exudation, and Degeneration Conference, highlighting the favorable safety data from the ongoing ZETA-1 Phase 2 trial of APX3330 in DR.

- In February 2022, Inder Paul Singh, MD, presented at theCataract Surgery: Telling It Like It Is Conference in Orlando. Dr. Singh presented the positive results from the

completed VEGA-1 Phase 2 trial of Nyxol in presbyopia as a single agent and in combination with adjunctive LDP.

- In January 2022, Mina Sooch, Founder and CEO participated in the panel discussion titled *"The Role of Gender Equality in Changing the Life Sciences Investment and Innovation Landscape"* at the 11th LifeSci Partners Corporate Access Event.

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

- In October 2021, the Company announced the publication of a review article titled "Inhibition of APE1/Ref-1 for Neovascular Eye Disease: From Biology to Therapy" in the Special Issue "Advances in Molecular Activity of Potential Drugs" of the *International Journal of Molecular Sciences.* 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.

- In October 2021, 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"). 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.

- In October 2021, Michael J. Allingham, MD, PhD presented at the39th Annual Scientific Meeting of the American Society of Retina Specialists (ASRS) (Diabetic Retinopathy 1 Symposium) held in San Antonio, highlighting the favorable safety and tolerability data for APX3330 in over 300 healthy volunteers and cancer/hepatitis patients across 11 Phase 1 and Phase 2 studies. In addition, Mina Sooch, CEO, presented APX3330 history and the design of the ongoing Phase 2 trial in DR at the OIS Retina Innovation Summit@ASRS on October 7, 2021 in San Antonio, TX.

Fourth Quarter and Year Ended December 31, 2021 Financial Highlights

As of December 31, 2021, the Company had cash and cash equivalents of approximately $24.5 million. Based on current projections, management believes the current cash on hand will be sufficient to fund operations into the second quarter of 2023. Net cash used in operating activities for the quarter and year ended December 31, 2021 was $5.6 million and $19.4 million, respectively.

No collaboration revenue was recorded in the fourth quarter. Collaborative revenue was $0.6 million for the year ended December 31, 2021. Revenue was derived from the collaboration and license agreements with Processa and Biosense related to certain Rexhan products and technology transfers. There was no collaboration revenue recognized during the comparable prior year periods.

General and administrative expenses for the quarter and year ended December 31, 2021 were $1.4 million and $8.1 million, respectively, compared to $1.3 million and $2.8 million for the quarter and year ended December 31, 2020, respectively. The $5.3 million increase for the year over year periods was primarily attributable to administrative employee headcount, stock-based compensation, insurance, legal and settlement costs, costs associated with operating as a public company subsequent to the reverse merger, and professional services and other operating costs. General and administrative expenses included $0.3 million and $0.2 million in non-cash stock-based compensation expense during the quarters ended December 31, 2021 and 2020, respectively, and $1.1 million and $0.7 million in non-cash stock-based compensation expense during the years ended December 31, 2021 and 2020, respectively.

Research and development expenses for the quarter and year ended December 31, 2021 were $4.7 million and $15.2 million, respectively, compared to $4.3 million and $6.6 million for the quarter and year ended December 31, 2020, respectively. The $8.5 million increase for the year over year periods was primarily attributable to clinical trial expense, manufacturing activities to support clinical advancement of Nyxol and APX3330, consulting services as well as regulatory and other research and development efforts. Research and development expenses also included $0.2 million and $0.3 million in non-cash stock-based compensation expense during the quarters ended December 31, 2021 and 2020, respectively, and $0.8 million in non-cash stock-based compensation expense during each of the years ended December 31, 2021 and 2020.

The loss from operations for the quarter ended December 31, 2021 was $6.2 million, compared to $14.0 million for the quarter ended December 31, 2020. The loss from operations for the year ended December 31, 2021 was $22.7 million, compared to $20.0 million for the year ended December 31, 2020. Net loss for the quarter ended December 31, 2021 was $6.3 million, compared to $18.7 million for the quarter ended December 31, 2020. Net loss for the year ended December 31, 2021 was $56.7 million, compared to $24.6 million for the year ended December 31, 2020. Net loss per share for the quarter ended December 31, 2021 was $0.35, compared to $2.46 for the quarter ended December 31, 2020. Net loss per share for the year ended December 31, 2021 was $3.82, compared to $5.28 for the year ended December 31, 2020.

The fair value change in derivative and warrant liabilities was a non-cash expense of zero for the quarter ended December 31, 2021 compared to a non-cash expense of $1.6 million for the quarter ended December 31, 2020. The fair value change in derivative and warrant liabilities was a non-cash expense of $33.8 million for the year ended December 31, 2021 compared to a non-cash expense of $1.5 million for the year ended December 31, 2020.

For further details on Ocuphire’s financial results, refer to the Company’s Annual Report on Form 10-K for the year ended December 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 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 9 completed clinical trials. Ocuphire reported positive top-line data for MIRA-2, the first Phase 3 registration trial for the treatment of RM, and recently initiated and completed enrollment in the second Phase 3 registration trial (MIRA-3) and pediatric safety trial (MIRA-4) in 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 low dose pilocarpine ("LDP") 0.4% as adjunctive therapy. The company recently completed enrollment in its Phase 3 study 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 enrolled second Phase 3 registration trial in RM (NCT05134974), MIRA-4 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, timing and results in RM, presbyopia, NVD and DR/DME future clinical trials as well as statements concerning the success and timing of planned regulatory filings and commercialization. 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 December 31,	
	2021	**2020**
Assets		
Current assets:		
Cash and cash equivalents	$ 24,534	$ 16,399
Prepaids and other assets	1,314	1,269
Short-term investments	219	—
Total current assets	26,067	17,668
Property and equipment, net	10	14
Total assets	$ 26,077	$ 17,682
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 1,584	$ 1,214
Accrued expenses	1,733	1,971
Short-term loan	538	—
Total current liabilities	3,855	3,185
Warrant liabilities	—	27,964
Total liabilities	3,855	31,149
Commitments and contingencies		
Stockholders' equity (deficit)		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of December 31, 2021 and 2020; no shares issued and outstanding at December 31, 2021 and 2020.	—	—

Common stock, par value $0.0001; 75,000,000 shares authorized as of December 31, 2021 and 2020; 18,845,828 and 10,882,495 shares issued and outstanding at December 31, 2021 and 2020, respectively.	2	1
Additional paid-in capital	111,588	19,207
Accumulated deficit	(89,368)	(32,675)
Total stockholders' equity (deficit)	22,222	(13,467)
Total liabilities and stockholders' equity (deficit)	$ 26,077	$ 17,682

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

	For the Quarter Ended December 31, (unaudited)		For the Year Ended December 31,	
	2021	**2020**	**2021**	**2020**
Collaborations revenue	$ —	$ —	$ 589	$ —
Operating expenses:				
General and administrative	$ 1,414	$ 1,310	$ 8,121	$ 2,818
Research and development	4,736	4,337	15,173	6,648
Acquired in-process research and development	—	8,376	—	10,502
Total operating expenses	6,150	14,023	23,294	19,968
Loss from operations	(6,150)	(14,023)	(22,705)	(19,968)
Interest expense	(2)	(5,425)	(2)	(6,847)
Fair value change in derivative and warrant liabilities	—	(1,644)	(33,829)	(1,486)
Gain on note extinguishment	—	2,412	—	3,672
Other (expense) income, net	(161)	—	(157)	9
Loss before income taxes	(6,313)	(18,680)	(56,693)	(24,620)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(6,313)	(18,680)	(56,693)	(24,620)
Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (6,313)	$ (18,680)	$ (56,693)	$ (24,620)
Net loss per share:				
Basic and diluted	$ (0.35)	$ (2.46)	$ (3.82)	$ (5.28)
Number of shares used in per share calculations:				
Basic and diluted	17,854,790	7,586,568	14,852,745	4,661,110

Source: Ocuphire Pharma