Opus Genetics Announces Financial Results for Third Quarter 2024 and Provides Corporate Update

In October, Ocuphire Pharma acquired Opus Genetics, creating a leading, clinical-stage company focused on the development of gene therapy treatments for rare inherited retinal diseases (IRDs)

The pro forma cash balance of the combined company was approximately $37 million as of September 30, 2024 (preliminary and unaudited), expected to extend runway into 2026

Four clinical data readouts expected in 2025

FARMINGTON HILLS, Mich., November 12, 2024 (GLOBE NEWSWIRE) – Opus Genetics, Inc. (Nasdaq: IRD), a clinical-stage ophthalmic biotechnology company developing gene therapies for the treatment of inherited retinal diseases (IRDs) and other ophthalmologic disorders, today announced financial results for the third quarter ended September 30, 2024 and provided a corporate update.

“In October 2024, we acquired Opus Genetics with the goal of creating a leading gene therapy franchise to treat inherited retinal diseases,” said George Magrath, M.D., now CEO of Opus Genetics. “The transaction expanded our pipeline substantially, adding compelling gene therapy assets. The most advanced of these new candidates, LCA5, has generated positive six-month proof-of-concept data in patients with advanced disease. A second candidate, OPGx-BEST1 targets one of the largest IRD populations, and we’re excited to begin dosing patients next year. We continue to develop Phentolamine Ophthalmic Solution 0.75% in new indications, and this franchise is expected to provide meaningful potential cashflow in the future, if approved for the new indications. Our expected cash runway has been extended into 2026, through expected efficacy readouts from four clinical programs in 2025, including Phase 3 studies in two indications for Phentolamine Ophthalmic Solution 0.75% and two studies for the new gene therapy clinical assets.”

Corporate Updates

Acquisition of Opus Genetics

- On October 22, Ocuphire acquired Opus Genetics, Inc., a clinical-stage gene therapy company for inherited retinal diseases (IRDs), in an all-stock transaction. The merger creates a transformative biotech company committed to being a leader

in the development of gene therapies for the treatment of IRDs. In connection with the merger, the combined company was renamed Opus Genetics, Inc., and began trading on Nasdaq under the ticker symbol “IRD,” effective as of October 24, 2024. As consideration for the Opus Acquisition, the Company issued 5,237,063 shares of its common stock and 14,145.374 shares of Series A Preferred Stock, each of which is convertible into 1,000 shares of common stock. As of November 7, 2024 there were 31,568,457 shares of the Company’s common stock outstanding. If the shares of Series A Preferred Stock were converted as of that date, there would be a total of 45,713,831 shares of common stock outstanding.

- As a combined company, we have an expanded pipeline that includes seven assets from our adeno-associated virus (AAV)-based gene therapy portfolio, each of them being developed for a specific IRD, as well as Phentolamine Ophthalmic Solution 0.75%, which is currently being evaluated in presbyopia and dim (mesopic) light vision disturbances (sometimes referred to as DLD) after keratorefractive surgery.
- Our most advanced gene therapy candidate, OPGx-LCA5, is being developed to treat LCA5, an early-onset, severe hereditary retinal diseases. An open-label, dose-escalation Phase 1/2 clinical trial is ongoing. The trial has shown early clinical proof-of-concept, with new six-month data demonstrating visual improvement in three out of three adult patients participating in the trial, each of whom has late-stage disease. Enrollment of the first pediatric patients is expected in the first quarter of 2025, with the first data anticipated in the third quarter of 2025.
- OPGx-BEST1 is in development for Bestrophin1-associated retinal disease. IND-enabling studies with OPGx-BEST1 have generated compelling proof-of-concept efficacy data and exhibited favorable safety. We anticipate filing a Clinical Trial Authorization (CTA) application in Germany in 2025 for commencement of a Phase 1/2 clinical trial.

<u>Phentolamine Ophthalmic Solution 0.75%</u>

- Enrollment is ongoing in the LYNX-2 Phase 3 registration study evaluating Phentolamine Ophthalmic Solution 0.75% for the treatment of dim light disturbances under mesopic (low) light conditions following keratorefractive surgery. Top-line data are expected in the first quarter of 2025. The LYNX-2 trial is being conducted under conditions of a Special Protocol Assessment (SPA) with the FDA.
- The VEGA-3 Phase 3 trial evaluating Phentolamine Ophthalmic Solution 0.75% for the treatment of presbyopia is also enrolling patients. Top-line results are expected in the first half of 2025.
- The Phentolamine Ophthalmic Solution 0.75% development portfolio is being funded by Ocuphire’s partner in both indications (presbyopia and dim light vision disturbances).

APX3330

- The Company intends to seek a strategic partner to advance further late-stage development of APX330, its novel, oral REF-1 inhibitor for diabetic retinopathy (DR), given the development timelines and capital requirements. In the meantime, discussions remain ongoing with the FDA regarding the SPA for a Phase 3 program in DR.

Financial Highlights for the Third Quarter Ended September 30, 2024

As of September 30, 2024, Ocuphire had cash and cash equivalents of $37 million. The pro forma cash balance of the combined company was approximately $37 million as of September 30, 2024 (preliminary and unaudited). Based on current projections, management believes that the cash on hand will be sufficient to fund operations into 2026.

License and collaborations revenue was $3.9 million and $11.9 million for the three months ended September 30, 2024 and 2023, respectively. Revenue during both quarterly periods was derived from the License Agreement. Revenue for the three months ended September 30, 2024 was comprised largely of the reimbursement of research and development services. The decrease compared to the corresponding prior year period was largely due to the one-time achievement of a $10.0 million milestone attributed to the FDA's approval of Phentolamine Ophthalmic Solution 0.75% for reversal of mydriasis in 2023.

Revenue for the three months ended September 30, 2024 also included an earned royalty payment in the amount of $14,000 from the sales of RYZUMVI, indicated for the treatment of pharmacologically-induced mydriasis by our commercial partner. Until further notice, we will report earned RYZUMVI royalties as a component of revenue listed in the Income Statement.

General and administrative expenses for the three months ended September 30, 2024 were $2.9 million compared to $2.1 million for the three months ended September 30, 2023. The increase period over period was primarily attributable to personnel-related costs, stock-based compensation, legal support costs and business development costs. These were offset in part by a reduction in non-legal professional service costs. General and administrative expenses included the following noncash items: $0.5 million and $0.3 million in stock-based compensation expense during both three months ended September 30, 2024 and 2023, respectively.

Research and development expenses for the three months ended September 30, 2024 were $9.0 million compared to $3.5 million for the three months ended September 30, 2023. The increase in the current period was primarily attributable to increased clinical costs related to the APX3330 development program and other research and development activities period over period, drug manufacturing costs and toxicology service costs related to APX3330, increased payroll related costs and regulatory and operating related expenses. Pursuant to the License Agreement, our budgeted research

and development expenses related to the development of Phentolamine Ophthalmic Solution 0.75%, are fully reimbursed by our development partner. Research and development expenses also included $0.2 million in stock-based compensation expense during each of the three-month periods ended September 30, 2024 and 2023.

Net loss for the quarter ended September 30, 2024, was $7.5 million or $(0.29) per basic and diluted share as compared to net income of $5.6 million or $0.26 and $0.25 per basic and diluted share, respectively, for the third quarter of 2023.

For further details on our financial results, including results for the nine-month period ended September 30, 2024, please refer to our Quarterly Report on Form 10-Q to be filed with the Securities and Exchange Commission.

About Opus Genetics

Opus Genetics is a clinical-stage ophthalmic biotechnology company developing gene therapies to treat patients with inherited retinal diseases (IRDs) and therapies to treat patients with other ophthalmologic disorders. The pipeline includes adeno-associated virus (AAV)-based gene therapies that address mutations in genes that cause different forms of bestrophinopathy, Leber congenital amaurosis (LCA) and retinitis pigmentosa. The company's most advanced gene therapy program is designed to address mutations in the LCA5 gene, which encodes the lebercilin protein and is currently being evaluated in a Phase 1/2 open-label, dose-escalation trial, with encouraging early data. BEST1 gene therapy is designed to address mutations in the BEST1 gene, which is associated with retinal degeneration; A Phase 1/2 study will be initiated in 2025. The pipeline also includes Phentolamine Ophthalmic Solution 0.75%, a non-selective alpha-1 and alpha-2 adrenergic antagonist to reduce pupil size, and APX3330, a novel small-molecule inhibitor of Ref-1 to slow the progression of non-proliferative diabetic retinopathy. Phentolamine Ophthalmic Solution 0.75% is currently being evaluated in Phase 3 trials for presbyopia and dim (mesopic) light vision disturbances. For more information, please visit www.opusgtx.com.

Forward Looking Statements

This press release contains 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 expectations regarding our cash runway, data from and future enrollment for our clinical trials, our pipeline of additional indications, expectations of potential growth, and our expectations regarding integration following the acquisition of Opus Genetics, including with respect to the combination of their portfolio of clinical assets into our existing portfolio and our combined focus on gene therapy treatment.

These forward-looking statements relate to us, our business prospects and our results of operations and are subject to certain risks and uncertainties posed by many factors

and events that could cause our actual business, prospects and results of operations to differ materially from those anticipated by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those described under the heading “Risk Factors” included in Ocuphire’s Annual Report on Form 10-K. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. In some cases, you can identify forward-looking statements by the following words: “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “aim,” “may,” “ongoing,” “plan,” “potential,” “predict,” “project,” “should,” “will,” “would” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. We undertake no obligation to revise any forward-looking statements in order to reflect events or circumstances that might subsequently arise.

These forward-looking statements are based upon our 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:

- The success and timing of regulatory submissions and pre-clinical and clinical trials, including enrollment and data readouts;
- Regulatory requirements or developments;
- Changes to or unanticipated events in connection with clinical trial designs and regulatory pathways;
- Delays or difficulties in the enrollment of patients in clinical trials;
- Substantial competition and rapid technological change;
- Our development of sales and marketing infrastructure;
- Future revenue losses and profitability;
- Our relatively short operating history;
- Changes in capital resource requirements;
- Risks related to our inability to obtain sufficient additional capital to continue to advance our product candidates and our preclinical programs;
- Domestic and worldwide legislative, regulatory, political and economic developments;
- Employee misconduct
- Reliance on third parties;
- Future, potential product liability and securities litigation;
- System failures, unplanned events, or cyber incidents;
- The substantial number of shares subject to potential issuance associated with our equity line of credit arrangement;
- Risks that our partnership or other licensing arrangements, may not facilitate the commercialization or market acceptance of our product candidates;
- Future fluctuations in the market price of our common stock;
- Our ability to realize the expected benefits of the acquisition of Opus Genetics;
- Our ability to execute clinical programs for gene therapies successfully and changes in expected commercial value we predict from the development of gene therapies;

- The success and timing of commercialization of any of our product candidates; and
- Obtaining and maintaining our intellectual property rights.

The foregoing review of important factors that could cause actual events to differ from expectations should not be construed as exhaustive. Readers are urged to carefully review and consider the various disclosures made by us in this report and in our other reports filed with the Securities and Exchange Commission that advise interested parties of the risks and factors that may affect our business. All forward-looking statements contained in this press release speak only as of the date on which they were made. We undertake no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

Contacts

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

	As of	
	September 30, 2024	**December 31, 2023**
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 36,632	$ 50,501
Accounts receivable	1,857	926
Contract assets and unbilled receivables	1,468	1,407
Prepaids and other assets	429	1,099
Short-term investments	3	15
Total current assets	40,389	53,948
Property and equipment, net	—	—
Total assets	$ 40,389	$ 53,948
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 844	$ 2,153
Accrued expenses	5,171	1,815
Derivative liability	74	74
Total current liabilities	6,089	4,042
Total liabilities	6,089	4,042
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, par value $0.0001; 10,000,000 shares authorized as of September 30, 2024 and December 31, 2023; no shares issued and outstanding at September 30, 2024 and December 31, 2023.	—	—
Common stock, par value $0.0001; 125,000,000 and 75,000,000 shares authorized as of September 30, 2024 and December 31, 2023, respectively; 26,198,444 and 23,977,491 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively.	3	2
Additional paid-in capital	138,160	131,370
Accumulated deficit	(103,863)	(81,466)
Total stockholders' equity	34,300	49,906
Total liabilities and stockholders' equity	$ 40,389	$ 53,948

Ocuphire Pharma, Inc.
Condensed 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,	
	2024	**2023**	**2024**	**2023**
License and collaborations revenue	$ 3,867	$ 11,935	$ 6,690	$ 17,358
Operating expenses:				
General and administrative	2,894	2,055	10,918	8,680
Research and development	8,982	3,494	19,817	13,812
Total operating expenses	11,876	5,549	30,735	22,492
(Loss) income from operations	(8,009)	6,386	(24,045)	(5,134)
Financing costs	—	(1,328)	—	(1,328)
Fair value change in derivative liability	—	61	—	61
Other income, net	483	456	1,648	1,224
(Loss) income before income taxes	(7,526)	5,575	(22,397)	(5,177)
Provision for income taxes	—	(14)	—	(14)
Net (loss) income	(7,526)	5,561	(22,397)	(5,191)
Other comprehensive (loss) income, net of tax	—	—	—	—
Comprehensive (loss) income	$ (7,526)	$ 5,561	$ (22,397)	$ (5,191)
Net (loss) income per share:				
Basic	$ (0.29)	$ 0.26	$ (0.88)	$ (0.25)
Diluted	$ (0.29)	$ 0.25	$ (0.88)	$ (0.25)
Number of shares used in per share calculations:				
Basic	26,145,080	21,446,648	25,501,117	21,117,211
Diluted	26,145,080	22,405,995	25,501,117	21,117,211