March 31, 2025

Opus Genetics Announces Financial Results for Full Year 2024

Provides update on the Company's transformation and promising portfolio of innovative gene therapy treatments for inherited retinal diseases

Strong cash position with $21.5 million financing to supplement $30.3 million year-end balance

New capital supports delivery on key milestones for two lead gene therapy candidates OPGx-LCA5 and OPGx-BEST1

RESEARCH TRIANGLE PARK, N.C., March 31, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. ("Opus" or the "Company") (Nasdaq: IRD), a clinical-stage ophthalmic biopharmaceutical company developing important new therapies for the treatment of inherited retinal diseases (IRDs) and other ophthalmic disorders, today announced financial results for the full year ended December 31, 2024, and provided a corporate update.

"2024 marked a year of significant progress and change for the Company," said George Magrath, M.D., Chief Executive Officer. "We began the year with an uncertain future, with a lead asset that had failed to meet its primary endpoints in Phase 2 and another asset that had been fully out-licensed for commercialization. Given this portfolio, we took decisive action to position the Company to build additional value. Our transformative acquisition of privately held Opus Genetics in October strengthens our pipeline with a promising portfolio of gene therapy assets."

"2025 is shaping up to be another exciting year," Dr. Magrath continued. "We recently completed a public offering and concurrent private placement, raising $21.5 million in capital primarily from leading institutional healthcare investors who share our confidence in our strategic direction. This financing strengthens our balance sheet and provides the resources necessary to achieve key milestones in our IRD programs."

Dr. Magrath continued, "We are now focused on executing the opportunities we have ahead of us and are excited about the advancement of our pipeline. Notably, in the 12-month results from our OPGx-LCA5 Phase 1/2 trial, we observed the continued durability of positive response observed at six months, which reinforced our confidence in the potential of this program. We look forward to sharing the key findings at the forthcoming annual meeting of the Association for Research in Vision and Ophthalmology (ARVO). In addition, we recently held a constructive Type D meeting with the U.S. Food and Drug Administration (FDA) to discuss trial design and registrational endpoints for OPGx-LCA5."

"Looking ahead, we see several near-term catalysts on the horizon. We expect to announce up to four clinical trial data readouts in 2025, including Phase 3 data on dim light vision

disturbances and in presbyopia for Phentolamine Ophthalmic Solution 0.75%. We also expect our second gene therapy candidate, OPGx-BEST1, to enter the clinic later this year. With a strengthened pipeline of promising gene therapies, a strong cash position, and a refreshed Board of Directors, I believe Opus is well positioned to advance its mission of improving the lives of patients suffering from IRDs," concluded Dr. Magrath.

Recent Business Highlights and Corporate Updates

Acquisition of Opus Genetics

- On October 22, 2024, we acquired privately-held Opus Genetics Inc., a clinical-stage gene therapy company focused on IRDs, in an all-stock transaction. In connection with the acquisition, the combined company adopted the name Opus Genetics, Inc., and began trading on Nasdaq under the ticker symbol "IRD," effective as of October 24, 2024.
- The Company's IRD assets are supported by cutting-edge science developed by gene therapy pioneers at the University of Pennsylvania, Harvard Medical School, and University of Florida.
- The combined company now has an expanded pipeline, which includes a portfolio of seven adeno-associated virus (AAV)-based gene therapy assets, each targeting a specific IRD, as well as Phentolamine Ophthalmic Solution 0.75%, which is currently being evaluated in presbyopia and mesopic (dim) light vision disturbances (sometimes referred to as DLD) after keratorefractive surgery.

Financing

- In March 2025, we completed an underwritten public offering, led by Perceptive Advisors and Nantahala Capital, with participation from other new institutional biotechnology investors, raising gross proceeds of $20 million. This was accompanied by a concurrent private placement, generating gross proceeds of $1.5 million.
- The financing increases the Company's available cash resources to approximately $50.7 million, extending our runway and providing the resources needed to achieve key upcoming milestones for our portfolio.
- Additionally, there is potential for up to $21.4 million in further proceeds upon the exercise of warrants.

Gene Therapy Programs

OPGx-LCA5

- Opus' most advanced investigational gene therapy candidate, OPGx-LCA5, is being developed to treat patients with inherited retinal degeneration due to biallelic mutations in the Leber congenital amaurosis 5 (LCA5)-related LCA, an early-onset, severe hereditary retinal degeneration. The candidate is currently being evaluated in an open-label Phase 1/2 clinical trial which has shown clinical proof-of-concept. One-year data has provided evidence that the therapy supported visual improvement in all three adult patients participating in the trial, each of whom has late-stage disease. Enrollment of the first pediatric patient occurred in the first quarter of 2025.
- A Type D meeting was held with the FDA in March 2025 to discuss the regulatory path for OPGx-LCA5, including the design of a potential registrational study. Opus will

continue to work with the FDA on the most appropriate trial design, including the primary endpoint.
- Six-month results from adult patients treated with OPGx-LCA5 were presented in a Key Opinion Leader webinar hosted by Opus on December 11, 2024. A replay of the webinar can be accessed here.

OPGx-BEST1

- OPGx-BEST1 is an investigational Phase 1/2-ready asset in development for IRDs associated with mutations in the BEST1 gene (sometimes referred to as “Best Disease”), which can lead to legal blindness.
- In IND-enabling studies with OPGx-BEST1, we have observed compelling safety and efficacy data in support of a first-in-human clinical trial.
- We anticipate commencement of a Phase 1/2 trial in 2025 and aim to obtain preliminary data by the first quarter of 2026.

Phentolamine Ophthalmic Solution 0.75%

- The LYNX-2 pivotal Phase 3 trial evaluating Phentolamine Ophthalmic Solution 0.75% for the treatment of decreased vision under low light conditions following keratorefractive surgery completed enrollment in the first quarter of 2025. The LYNX-2 trial is covered by a Special Protocol Assessment (“SPA”) agreement with the FDA, which ensures agreement with the FDA on the trial design, endpoints, and study size (power).
- The FDA granted Fast Track designation for Phentolamine Ophthalmic Solution 0.75% for treatment of significant chronic night driving impairment with concomitant increased risk of motor vehicle accidents and debilitating loss of best spectacle corrected mesopic vision in keratorefractive patients with photic phenomena (i.e., glare, halos, starburst).
- The VEGA-3 pivotal Phase 3 clinical trial evaluating Phentolamine Ophthalmic Solution 0.75% for the treatment of presbyopia completed enrollment in the first quarter of 2025 with topline data expected in the first half of 2025.
- The development portfolio related to Phentolamine Ophthalmic Solution 0.75% is being funded by our partner, Viatris Inc. (“Viatris”), in both indications (presbyopia and dim light vision disturbances).

APX3330

- In December 2024, we reached agreement with the FDA under a SPA for a Phase 3 clinical trial evaluating APX3330, a novel, oral REF-1 inhibitor for the treatment of moderate to severe non-proliferative diabetic retinopathy (NPDR). The SPA agreement reflects alignment on a proposed Phase 3 trial design, endpoints, and planned analyses to support submission of a New Drug Application for treatment of NPDR.
- We intend to seek a strategic partner to advance late-stage development of APX3330.

Expected potential growth drivers in 2025 and beyond

- Three abstracts on our investigational gene therapy candidates have been accepted for presentation at the ARVO 2025 Meeting, to take place from May 4 to 8, 2025 in Salt Lake City, UT. These include a presentation on 12-month data from the first three adult

patients in the ongoing Phase 1/2 trial of OPGx-LCA5.
- Data on the first pediatric patient in the trial for OPGx-LCA5 data are anticipated in the third quarter of 2025.
- Initiation of a Phase 1b/2a clinical trial for OPGx-BEST1 is planned for 2025. We aim to obtain preliminary data by the first quarter of 2026.
- Topline date from the LYNX-2 pivotal Phase 3 trial evaluating Phentolamine Ophthalmic Solution 0.75% for decreased vision under low light conditions following keratorefractive surgery are expected mid-year 2025.
- Topline data from the VEGA-3 Phase 3 clinical trial evaluating Phentolamine Ophthalmic Solution 0.75% for the treatment of presbyopia are expected in the first half of 2025.

Financial Highlights for the Full Year Ended December 31, 2024

As of December 31, 2024, Opus had cash and cash equivalents of $30.3 million. Based on current projections, management believes that the cash on hand will be sufficient to fund operations into the second half of 2026.

License and collaborations revenue was $11.0 million for the year ended December 31, 2024 compared to $19.0 million for the year ended December 31, 2023. Revenue during 2024 and 2023 was derived from the output of research and development services in connection with the Viatris License Agreement. Additionally, revenue during 2023 was derived in part from a milestone payment of $10.0 million attributed to the FDA's approval of Phentolamine Ophthalmic Solution 0.75%, under brand name RYZUMVI for the treatment of pharmacologically induced mydriasis.

General and administrative expenses for the year ended December 31, 2024 were $18.2 million compared to $12.0 million for the year ended December 31, 2023. The increase was attributed to transaction costs in connection with the Opus Acquisition, payroll related costs, legal support, business development activities, and other costs. General and administrative expenses included $2.4 million in stock-based compensation expense during each of the years ended December 31, 2024 and 2023.

Research and development expenses for the year ended December 31, 2024 were $26.9 million compared to $17.7 million for the year ended December 31, 2023. The increase was primarily attributable to clinical costs related to the LYNX-2 and VEGA-3 trials and other research and development activities period over period, drug manufacturing costs, and toxicology service costs related to APX3330, payroll related costs, and regulatory and operating related expenses. Pursuant to the license agreement with "Viatris", the Company's budgeted research and development expenses related to the development of Phentolamine Ophthalmic Solution 0.75%, are fully reimbursed by Viatris. Research and development expenses included $1.0 million and $1.1 million in stock-based compensation expense during the years ended December 31, 2024 and 2023, respectively.

Research and development projects of the Acquired Company, which were in-process at the closing of the Opus Acquisition were expensed as acquired in-process research and development ("IP R&D") amounting to $28.0 million. There were no IP R&D costs in the comparable prior year period.

Net loss for the year ended December 31, 2024, was $57.5 million or ($2.15) per basic and

diluted share as compared to net loss of $10.0 million or ($0.46) per basic and diluted share for 2023.

For further details on our financial results, refer to the Company's Annual Report on Form 10-K to be filed with the Securities and Exchange Commission.

About Opus Genetics

Opus Genetics is a clinical-stage ophthalmic biopharmaceutical company developing therapies to treat patients with inherited retinal diseases (IRDs) and other treatments for ophthalmic disorders. Our pipeline includes adeno-associated virus (AAV)-based investigational gene therapies that address mutations in genes that cause different forms of bestrophinopathy, Leber congenital amaurosis (LCA) and retinitis pigmentosa. Our most advanced investigational 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. Our pipeline also includes OPGx-BEST1 investigational gene therapy, designed to address mutations in the *BEST1* gene, which is associated with retinal degeneration. The pipeline also includes Phentolamine Ophthalmic Solution 0.75%, a non-selective alpha-1 and alpha-2 adrenergic antagonist being investigated to reduce pupil size, and APX3330, a novel small-molecule inhibitor of Ref-1 being investigated 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. We have reached agreement with the FDA under SPA for a Phase 3 trial to evaluate oral APX3330 for the treatment of DR 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, expectations regarding our cash runway, expectations of potential growth, expectations regarding integration following our acquisition of privately-held Opus Genetics Inc., including with respect to the combination of their portfolio of clinical assets into our existing portfolio and our combined focus on gene therapy treatment, and statements concerning data from and future enrollment for our clinical trials and our pipeline of additional indications.

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 our Quarterly Report on Form 10-Q for the quarter ended September 30, 2024 and in our other filings with the U.S. Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this press release. 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:

- Failure to successfully integrate our businesses with Former Opus could have a material adverse effect on our business, financial condition and results of operations;
- The Opus Acquisition significantly expanded our product pipeline and business operations and shifted our business strategies, which may not improve the value of our common stock;
- Our gene therapy product candidates are based on a novel technology that is difficult to develop and manufacture, which may result in delays and difficulties in obtaining regulatory approval;
- Our planned clinical trials may face substantial delays, result in failure, or provide inconclusive or adverse results that may not satisfy FDA requirements to further develop our therapeutic products;
- Changes in regulatory requirements could result in increased costs or delays in development timelines;
- We depend heavily on the success of our product pipeline; if we fail to find strategic partners or fail to adequately develop or commercialize our pipeline products, our business will be materially harmed;
- Others may discover, develop, or commercialize products similar to those in our pipeline before or more successfully than we do or develop generic variants of our products even while our product patents remain active, thereby reducing our market share and potential revenue from product sales;
- We do not currently have any sales or marketing infrastructure in place and we have limited drug research and discovery capabilities;
- The future commercial success of our products could significantly depend upon several uncertain factors, including third-party reimbursement practices and the existence of competitors with similar products;
- Product liability lawsuits against us or our suppliers or manufacturers could cause us to incur substantial liabilities and could limit commercialization of any product candidate that we may develop;
- Failure to comply with health and safety laws and regulations could lead to material fines;
- We have not generated significant revenue from sales of any products and expect to incur losses for the foreseeable future;
- Our future viability is difficult to assess due to our short operating history and our future need for substantial additional capital, which could be limited by any adverse developments that affect the financial services industry;
- Raising additional capital may cause our stockholders to be diluted, among other adverse effects;
- We operate in a highly regulated industry and face many challenges complying to sudden changes in legislative reform or the regulatory environment, which affects our pipeline stability and could impair our ability to compete in international markets;
- We may not receive regulatory approval to market our developed product candidates within or outside of the U.S.;

- With respect to any of our product candidates that receive marketing approval, we may be subject to substantial penalties if we fail to comply with applicable regulatory requirements;
- Our potential relationships with healthcare providers and third-party payors will be subject to certain healthcare laws and regulations, which could expose us to extensive potential liabilities;
- We rely on third parties for material aspects of our business, such as conducting our nonclinical and clinical trials and supplying and manufacturing bulk drug substances, which exposes us to certain risks;
- We may be unsuccessful in entering into or maintaining licensing arrangements (such as the Viatris License Agreement) or establishing strategic alliances on favorable terms, which could harm our business;
- Our current focus on the cash-pay utilization for future sales of RYZUMVI may limit our ability to increase sales or achieve profitability with this product;
- Inadequate patent protection for our product candidates may result in our competitors developing similar or identical products or technology, which would adversely affect our ability to successfully commercialize;
- We may be unable to obtain full protection for our intellectual property rights under U.S. or foreign laws;
- We may become involved in lawsuits for a variety of reasons associated with our intellectual property rights, including alleged infringement suits initiated by third parties;
- We are dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy;
- As we grow, we may not be able to operate internationally or adequately develop and expand our sales, marketing, distribution, and other corporate functions, which could disrupt our operations;
- The market price of our common stock is expected to be volatile and subject to certain dilutive risks associated with our Equity Line of Credit arrangement; and
- Factors out of our control related to our securities, such as securities litigation or actions of activist stockholders, could adversely affect our business and stock price and cause us to incur significant expenses.

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

Corporate	Investor Relations
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Opus Genetics, Inc.
Consolidated Balance Sheets
(in thousands, except share amounts and par value)

	As of December 31,	
	2024	**2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 30,321	$ 50,501
Accounts receivable	3,563	926
Contract assets and unbilled receivables	2,209	1,407
Prepaids and other current assets	515	1,099
Short-term investments	2	15
Total current assets	36,610	53,948
Property and equipment, net	252	—
Total assets	$ 36,862	$ 53,948
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,148	$ 2,153
Accrued expenses	8,145	1,815
Derivative liability	2	74
Total current liabilities	11,295	4,042
Total liabilities	11,295	4,042
Commitments and contingencies		
Series A preferred stock, par value $0.0001; 14,146 shares and no shares were designated as of December 31, 2024 and 2023, respectively; 14,145.374 and no shares issued and outstanding at December 31, 2024 and 2023, respectively; no liquidation preference as of December 31, 2024 and 2023.	18,843	—
Stockholders' equity:		
Preferred stock, par value $0.0001; 9,985,854 and 10,000,000 shares authorized as of December 31, 2024 and 2023, respectively; no shares issued and outstanding at December 31, 2024 and 2023.	—	—

Common stock, par value $0.0001; 125,000,000 and 75,000,000 shares authorized as of December 31, 2024 and 2023, respectively; 31,574,657 and 23,977,491 shares issued and outstanding at December 31, 2024 and 2023, respectively.	3	2
Additional paid-in capital	145,719	131,370
Accumulated deficit	(138,998)	(81,466)
Total stockholders' equity	6,724	49,906
Total liabilities, series A preferred stock and stockholders' equity	$ 36,862	$ 53,948

Opus Genetics, Inc.
Consolidated Statements of Comprehensive Loss
(in thousands, except share and per share amounts)

	For the Year Ended December 31,	
	2024	**2023**
License and collaborations revenue	$ 10,992	$ 19,049
Operating expenses:		
General and administrative	18,215	11,959
Research and development	26,851	17,653
Acquired in-process research and development	28,000	—
Total operating expenses	73,066	29,612
Loss from operations	(62,074)	(10,563)
Financing costs	—	(1,328)
Fair value change in derivative liabilities	72	80
Other income, net	4,470	1,837
Loss before income taxes	(57,532)	(9,974)
Provision for income taxes	—	(12)
Net loss	(57,532)	(9,986)
Other comprehensive loss, net of tax	—	—
Comprehensive loss	$ (57,532)	$ (9,986)
Net loss per share:		
Basic and diluted	$ (2.15)	$ (0.46)
Number of shares used in per share calculations:		
Basic and diluted	26,715,526	21,589,821

Source: Opus Genetics, Inc.