May 15, 2025

Opus Genetics Announces Financial Results for First Quarter 2025 and Provides Corporate Update

ARVO presentation highlights 12-month results from Phase 1/2 study that support potential of OPGx-LCA5 gene therapy to restore meaningful vision

Pediatric cohort of LCA5 study ongoing with initial multi-patient data anticipated in Q3 2025

OPGx-BEST1 on track for IND filing and initiation of Phase 1/2 trial with early data expected in Q1 2026

Leading healthcare investors provide funding to deliver on key milestones

RESEARCH TRIANGLE PARK, N.C., May 15, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. (Nasdaq: IRD), a clinical-stage ophthalmic biopharmaceutical company developing important new therapies for the treatment of inherited retinal diseases (IRDs) and other ophthalmic disorders ("Opus" or the "Company"), today announced financial results for the first quarter ended March 31, 2025, and provided a corporate update.

"Opus is off to a strong start in 2025, with progress across our two product portfolios – the inherited retinal disease platform and the phentolamine eye drop franchise," said George Magrath, M.D., Chief Executive Officer. "We reported positive 12-month data from the Phase 1/2 trial of OPGx-LCA5 for the treatment of Leber congenital amaurosis 5 (LCA5), an inherited disease which leads to severe vision loss from infancy. The evidence of durable efficacy in adult patients is very encouraging, with the treatment benefits previously seen at 6 months now sustained out to one year. We are now enrolling pediatric patients and are encouraged by the meaningful improvement in visual function and early safety profile observed in the first of these patients at one month."

Dr. Magrath continued, "We believe that the initial success with our lead IRD program, OPGx-LCA5, has the potential to translate to the rest of our pipeline, which includes gene therapy candidates for six additional IRDs. We plan to initiate a Phase 1/2 study with our BEST-1 program later this year with early data expected in the first quarter of 2026.

"In the phentolamine eye drop pipeline, we look forward to near-term clinical data readouts from the Phase 3 data trials in dim light vision disturbances and in presbyopia. We ended the first quarter with a strong cash position, having completed a successful financing with leading institutional healthcare investors who share our confidence in the Company's strategic direction," concluded Dr. Magrath.

Strategic Highlights

- Opus's pipeline 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 (DLD) after keratorefractive surgery.
- Emerging clinical data on OPGx-LCA5 provide evidence of clinical proof of concept and support the potential of this novel gene therapy to restore meaningful vision for individuals with mutations in the LCA5 gene.
- Another gene therapy candidate, OPGx-BEST1, which Opus is developing for treatment of bestrophin-1 (BEST1)-related IRD, is on track to enter the clinic by the fourth quarter of 2025, with preliminary data expected in the first quarter of 2026.
- In March 2025, Opus completed a successful underwritten public offering and concurrent private placement anchored by leading healthcare investors, Perceptive Advisors and Nantahala Capital. The transaction raised approximately $21.5 million in gross proceeds with the potential for up to $21.4 million in additional proceeds upon exercise of warrants that are tied to data release from the BEST1 program.

Recent Business Highlights and Corporate Updates

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 LCA5 gene, an early-onset, severe hereditary retinal degeneration.
- One-year open-label data on adult patients being treated in the ongoing Phase 1/2 clinical trial of OPGx-LCA5 were featured in an oral presentation by Dr. Tomas Aleman of the Scheie Eye Institute, University of Pennsylvania, at the Association for Research in Vision and Ophthalmology (ARVO) annual meeting, in Salt Lake City. All treated patients had late-stage disease. Improvements in subjective and objective measures of efficacy that had been observed at six months persisted for one year.
- Enrollment in a cohort of three pediatric patients in the Phase 1/2 trial began in February 2025. Preliminary data on the first patient showed an encouraging early safety profile and meaningful improvement in visual function at one month. The pediatric cohort is expected to complete enrollment in the second quarter of 2025, with initial data from all three patients anticipated in the third quarter of 2025.
- The U.S. Food and Drug Administration (the "FDA") granted a Regenerative Medicine Advanced Therapy ("RMAT") designation to OPGx-LCA5 based on the early data from the first three patients treated. The RMAT designation program offers the potential for expedited development and review of regenerative medicine therapies that demonstrate the potential to address serious or life-threatening diseases based on preliminary clinical evidence. The designation provides sponsors with early interactions with the FDA, guidance on efficient development and manufacturing, and the opportunity to discuss surrogate endpoints to support accelerated approval.
- A Type D meeting was held with the FDA in March 2025 to discuss the potential 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 design, including the

primary endpoint.

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 of OPGx-BEST1 provided safety and efficacy data in support of a first-in-human clinical trial.
- Opus plans to file an IND and begin a Phase 1/2 trial by the fourth quarter of 2025, with preliminary data expected in 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 visual loss in low light conditions associated with keratorefractive surgery completed enrollment in the first quarter of 2025 with topline data expected mid-year 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 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 the Company’s partner, Viatris Inc., in both indications (presbyopia and dim light vision disturbances).

Expected Growth Drivers in 2025 and Beyond

- Initial data from three pediatric patients treated with OPGx-LCA5 anticipated in Q3 2025.
- IND filing and initiation of a Phase 1/2 clinical trial for OPGx-BEST1 is planned for 2025, with preliminary data expected in Q1 2026.
- Topline data from the LYNX-2 pivotal Phase 3 trial evaluating Phentolamine Ophthalmic Solution 0.75% for visual loss in low light conditions associated with keratorefractive surgery are expected mid-year 2025.
- Topline data from the VEGA-3 pivotal 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 First Quarter Ended March 31, 2025

As of March 31, 2025, Opus had cash and cash equivalents of $41.8 million. Based on current projections, management believes that the cash on hand will be sufficient to fund operations into the second quarter of 2026.

License and collaborations revenue was $4.4 million and $1.7 million during the three months ended March 31, 2025 and 2024, respectively. Revenue during both quarterly periods was derived from the Company's license and collaboration agreement (the "Viatris License Agreement") with Viatris, Inc. ("Viatris") largely from the reimbursement of research and development services and to a much lesser degree from royalty payments stemming from the sales of RYZUMVI™ by Viatris.

General and administrative ("G&A") expenses for the three months ended March 31, 2025 were $6.3 million compared to $4.7 million for the three months ended March 31, 2024. The increase was primarily attributable to professional services fees, corporate legal support, legal fees associated with intellectual property and public company costs, including filing fees and investor relations and governance costs, offset in part by decreases in general operating and other costs on a net basis. G&A expenses included $0.6 million and $0.8 million in stock-based compensation expense during the three months ended March 31, 2025 and 2024, respectively.

Research and development ("R&D") expenses for the three months ended March 31, 2025 were $8.0 million compared to $4.7 million for the three months ended March 31, 2024. The increase was primarily attributable to higher clinical costs and payroll related costs, offset partially by lower manufacturing expenses attributed to an activity reduction in the VEGA-2 trial and by lower regulatory and other costs on a net basis. Pursuant to the Viatris License Agreement, budgeted R&D expenses related to the development of the Phentolamine Ophthalmic Solution 0.75% products have been fully reimbursed by Viatris to date. R&D expenses included $0.3 million and $0.2 million in stock-based compensation expense during the three months ended March 31, 2025 and 2024, respectively.

Net loss for the quarter ended March 31, 2025, was $8.2 million or $(0.24) per basic and diluted share, as compared to net loss of $7.1 million, or $(0.29) per basic and diluted share, for the quarter ended March 31, 2024.

For further details on financial results, refer to the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2025 to be filed with the Securities and Exchange Commission (the "SEC").

About Opus Genetics

Opus Genetics is a clinical-stage ophthalmic biopharmaceutical company developing therapies to treat patients with IRDs and other treatments for ophthalmic disorders. Our pipeline includes adeno-associated virus (AAV)-based investigational gene therapies that address gene mutations responsible for different forms of Leber congenital amaurosis ("LCA"), bestrophinopathy 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 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 that is currently being evaluated in Phase 3 trials for presbyopia and mesopic (dim) light vision disturbances. For additional information, please visit www.opusgtx.com.

University of Pennsylvania ("Penn") Financial Disclosure: The laboratory of Dr. Tomas Aleman has received clinical trial research funding from Opus Genetics. Penn and Dr. Aleman have either received, or may receive in the future, financial consideration related to the licensing of certain Penn intellectual property to Opus Genetics.

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, 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2024, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, and in our other filings with the SEC. 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:

- Our clinical data related to gene therapies for the treatment of IRDs are preliminary and related to a relatively small group of patients, and, as a result, data that initially appear promising may be revised, updated, or invalidated at a later data readout and/or may ultimately not be capable of duplication in additional patients;

- Failure to successfully integrate our businesses following our acquisition of former Opus Genetics Inc. (the "Opus Acquisition") 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;

- Delays or difficulties associated with patient enrollment in clinical trials may affect our ability to conduct and complete those clinical trials and obtain necessary regulatory approvals;

- 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, access to which could be limited by any adverse developments that affect the financial services market;

- 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 adapting 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;
- Our common stock may be subject to delisting from the Nasdaq Capital Market and delisting could adversely affect our ability to access capital markets;
- 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; and
- Impact from current or proposed tariffs on imported goods we purchase.

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 SEC 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.

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

	As of	
	March 31, 2025	**December 31, 2024**
Assets	(Unaudited)	
Current assets:		
Cash and cash equivalents	$ 41,792	$ 30,321
Accounts receivable	3,080	3,563
Contract assets and unbilled receivables	1,675	2,209
Prepaids and other current assets	1,380	515
Short-term investments	1	2
Total current assets	47,928	36,610
Property and equipment, net	239	252
Total assets	$ 48,167	$ 36,862
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,430	$ 3,148
Accrued expenses and other liabilities	9,106	8,147
Warrant liabilities	12,715	—
Total current liabilities	24,251	11,295
Total liabilities	24,251	11,295
Commitments and contingencies		

Series A preferred stock, par value $0.0001; 14,146 shares were designated as of March 31, 2025 and December 31, 2024; 14,145.374 shares issued and outstanding at March 31, 2025 and December 31, 2024.	18,843	18,843
Stockholders' equity:		
Preferred stock, par value $0.0001; 9,985,854 shares authorized as of March 31, 2025 and December 31, 2024; no shares issued and outstanding at March 31, 2025 and December 31, 2024.	—	—
Common stock, par value $0.0001; 125,000,000 shares authorized as of March 31, 2025 and December 31, 2024; 45,483,823 and 31,574,657 shares issued and outstanding at March 31, 2025 and December 31, 2024, respectively.	5	3
Additional paid-in capital	152,260	145,719
Accumulated deficit	(147,192)	(138,998)
Total stockholders' equity	5,073	6,724
Total liabilities, series A preferred stock, and stockholders' equity	$ 48,167	$ 36,862

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

	Three Months Ended March 31,	
	2025	**2024**
License and collaborations revenue	$ 4,370	$ 1,711
Operating expenses:		
General and administrative	6,346	4,670
Research and development	7,953	4,749
Total operating expenses	14,299	9,419
Loss from operations	(9,929)	(7,708)
Financing costs	(1,372)	—
Fair value change in warrant liabilities	2,805	—
Other income, net	302	602
Loss before income taxes	(8,194)	(7,106)
Benefit (provision) for income taxes	—	—
Net loss	(8,194)	(7,106)

Other comprehensive loss, net of tax	—	—
Comprehensive loss	$ (8,194)	$ (7,106)
Net loss per share:		
Basic and diluted	$ (0.24)	$ (0.29)
Number of shares used in per share calculations:		
Basic and diluted	33,884,920	24,520,475

Source: Opus Genetics, Inc.