November 12, 2025

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

- Positive 3-month pediatric and 18-month adult clinical data from OPGx-LCA5 Phase 1/2 trial support the potential for restoring cone-mediated vision -

- Successful FDA RMAT meeting provides the potential for an accelerated regulatory pathway to approval for OPGx-LCA5 -

- OPGx-BEST1 gene therapy program underway with recruitment ongoing in Phase 1/2 trial for the treatment of BEST1 disease -

- Supplemental New Drug Application submission planned by year-end 2025 for Phentolamine Ophthalmic Solution 0.75% for the treatment of presbyopia -

- Strengthened capital position from recent equity offering and non-dilutive funding from patient advocacy groups -

RESEARCH TRIANGLE PARK, N.C., Nov. 12, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. (Nasdaq: IRD) (the "Company" or "Opus Genetics"), a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases (IRDs), today announced financial results for the third quarter ended September 30, 2025, and provided a corporate update.

"We have taken critical steps in advancing our pipeline, including the positive data and successful outcome of our recent FDA meeting regarding our LCA5 program and the opening of recruitment in our BEST1 Phase 1/2 clinical trial," said George Magrath, M.D., Chief Executive Officer, Opus Genetics. "We look forward to continuing this momentum into next year, when we expect multiple data readouts across several programs. The financing we recently completed reinforces the strong support from existing shareholders and new prominent healthcare investors and provides us with financial resources to expedite the path toward multiple approvals."

Pipeline Updates

OPGx-LCA5 – Gene Therapy for Leber Congenital Amaurosis (LCA)

- In September 2025, positive data was reported from the six participants treated to date in an open-label, Phase 1/2 clinical trial. Large gains in cone-mediated vision with improvements across multiple measures of visual function were observed in the three

pediatric participants treated over three months. In the three adult participants, responses have been observed out to 18 months, underscoring the potential durability of the treatment response. OPGx-LCA5 has been well tolerated with no ocular serious adverse events or dose-limiting toxicities to date.
- Successful completion of a Type B Regenerative Medicine Advanced Therapy (RMAT) meeting with the U.S. Food and Drug Administration (FDA) provides the potential for an accelerated regulatory pathway to approval of OPGx-LCA5.
- The Company plans to advance its ongoing trial into a Phase 3 portion which is expected to enroll as few as 8 participants in a single arm, 12-month study utilizing an adaptive design, which provides flexibility on endpoints and number of participants, reflective of LCA5 as a rare condition with an urgent medical need.
- The first participant was enrolled in the planned run-in period of the Phase 3 portion of the study to evaluate the natural history of each participant to serve as their own control.
- Dosing with OPGx-LCA5 is anticipated in the second half of 2026 following availability of validated clinical drug supply manufactured with the intended commercial processes, with topline clinical data expected approximately one year later.
- LCA5 program featured on *Good Morning America* in honor of World Blindness Awareness Month.

OPGx-BEST1 – Gene Therapy for BEST1-Related IRD

- In August 2025, the FDA accepted the Company's Investigational New Drug (IND) application for OPGx-BEST1 to initiate a clinical trial.
- The Company is currently recruiting participants in an adaptive, open-label, dose-exploring Phase 1/2 trial known as BIRD1 to evaluate the safety and tolerability of subretinally injected OPGx-BEST1 in participants with Best Vitelliform Macular Dystrophy (BVMD) or Autosomal-Recessive Bestrophinopathy (ARB).
- Initial data is expected in the first quarter of 2026.

OPGx-RDH12 and OPGx-MERTK – Advancing with Non-Dilutive Support

- Partnership with the Global RDH12 Alliance provides up to $1.6 million in non-dilutive funding to accelerate development of OPGx-RDH12 for Leber congenital amaurosis (RDH12-LCA).
- Non-dilutive funding of up to $2 million received from the Retinal Degeneration Fund to advance OPGx-MERTK, targeting retinitis pigmentosa caused by pathogenic variants in the Mer proto-oncogene tyrosine kinase (MERTK) gene.

Phentolamine Ophthalmic Solution 0.75% (PS) – Advancing Toward Supplemental New Drug Application (sNDA) Submissions

- Based on positive clinical data from the VEGA-3 Phase 3 trial to treat presbyopia, Opus Genetics plans to submit an sNDA to the FDA by year-end 2025.
- Full recruitment has been completed in LYNX-3, the second pivotal Phase 3 trial in keratorefractive participants with visual disturbances under mesopic, low-contrast conditions, with topline results expected in the first half of 2026.
- The program is being conducted under a Special Protocol Assessment (SPA) and has received Fast Track Designation from the FDA.

Medical Publications and Presentations

- Peer-reviewed publication of LCA5 Phase 1/2 trial data in*Molecular Therapy*: "Recovery of Cone-Mediated Vision in a Severe Ciliopathy after Gene Augmentation: One-Year Results of a Phase I/II Trial for LCA5-LCA," authored by Tomas S. Aleman, M.D., et al.
- Presentation at Eyecelerator at the American Academy of Ophthalmology (AAO) Annual Meeting titled "Transformative Gene Therapies for the Treatment of Rare Inherited Retinal Diseases."
- Presentation at the Cell and Gene Meeting on the Mesa titled: "Transformative Gene Therapies for the Treatment of Rare Inherited Retinal Diseases."
- Poster presentation at the American Academy of Optometry Annual Meeting titled: "LYNX-2: A Pivotal Phase 3 Trial of Phentolamine Ophthalmic Solution in Post-Keratorefractive Surgery Subjects with Decreased Mesopic Visual Acuity."

Financial Results for the Third Quarter Ended September 30, 2025

Cash Position: As of September 30, 2025, Opus Genetics had cash and cash equivalents of $30.8 million. Subsequent to the end of the quarter, the Company raised approximately $23.0 million in gross proceeds through a registered direct offering of equity securities. Based on current operating plans, the Company expects its existing cash resources will fund operations into the second half of 2027, excluding any potential proceeds from callable warrants or future milestone payments. The existing cash position of over $50 million is expected to support progress through key milestones including initial data from the BEST1 program and running the pivotal LCA5 trial.

Revenue: License and collaborations revenue totaled $3.1 million for the third quarter of 2025, compared to $3.9 million in the same period in 2024. Revenue in both periods was driven by the Company's collaboration with Viatris, Inc. ("Viatris"), primarily from reimbursement of research and development (R&D) services. The decrease was due to lower PS R&D services.

General and Administrative (G&A) Expenses: G&A expenses were $5.0 million for the third quarter of 2025, compared to $2.9 million for the same period in 2024. The increase was primarily attributable to higher legal and patent-related costs, payroll and public company-related costs, and professional service fees. G&A expenses included $0.6 million and $0.5 million in stock-based compensation in the third quarters of 2025 and 2024, respectively.

Research and Development (R&D) Expenses: R&D expenses were $6.4 million for the third quarter of 2025, compared to $9.0 million for the same period in 2024. The decrease was primarily attributable to lower clinical research, manufacturing and toxicology costs for APX 3330 and PS, partially offset by increased IRD program expense. R&D expenses related to PS were fully reimbursed under the license and collaboration agreement with Viatris. R&D expenses included $0.2 million in stock-based compensation in both of the third quarters of 2025 and 2024.

Net Loss: Net loss for the third quarter of 2025 was $17.5 million, or $(0.25) per basic and diluted share, compared to a net loss of $7.5 million, or $(0.29) per basic and diluted share, for the third quarter of 2024. The increased net loss was primarily due to the fair value

change in warrant and other derivative liabilities related to the warrants issued in the March 2025 public offering and the March 2025 private placement, based on fluctuations in common stock fair value and underlying changes in volatility, expected term and interest rates.

About Opus Genetics

Opus Genetics is a clinical-stage biopharmaceutical company developing gene therapies to restore vision and prevent blindness in patients with inherited retinal diseases (IRDs). The Company is developing durable, one-time treatments designed to address the underlying genetic causes of severe retinal disorders. The Company's pipeline includes seven AAV-based programs, led by OPGx-LCA5 for LCA5-related mutations and OPGx-BEST1 for BEST1-related retinal degeneration, with additional candidates targeting RHO, RDH12, and MERTK. Opus Genetics is also advancing Phentolamine Ophthalmic Solution 0.75%, an approved small-molecule therapy for pharmacologically induced mydriasis, with additional indications in late-stage development for presbyopia and low-light visual disturbances following keratorefractive surgery. The Company is based in Research Triangle Park, NC. For more information, 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 related to cash runway, the clinical development, clinical results, preclinical data, and future plans for Phentolamine Ophthalmic Solution 0.75%, OPGx-LCA5, OPGx-BEST1, RDH12, and earlier stage programs, and expectations regarding 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 subsequent Quarterly Report on Form 10-Q, and 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. 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. 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.

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-Financial Tables Follow-

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

	As of	
	September 30, 2025	**December 31, 2024**
Assets	(Unaudited)	
Current assets:		
Cash and cash equivalents	$ 30,815	$ 30,321
Accounts receivable	2,916	3,563
Contract assets and unbilled receivables (Note 11)	1,364	2,209
Prepaids and other current assets	815	515
Short-term investments	—	2
Total current assets	35,910	36,610
Property and equipment, net	212	252
Total assets	$ 36,122	$ 36,862
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,395	$ 3,148
Accrued expenses and other liabilities	5,367	8,147
Warrant liabilities	21,325	—
Total current liabilities	29,087	11,295
Long-term funding agreement, related party	1,068	—
Total liabilities	30,155	11,295
Commitments and contingencies (Note 4 and Note 10)		
Series A preferred stock, par value $0.0001; 14,146 shares were designated as of September 30, 2025 and December 31, 2024; zero and 14,145.374 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively.	—	18,843

Stockholders' equity:		
Preferred stock, par value $0.0001; 9,985,854 shares authorized as of September 30, 2025 and December 31, 2024; no shares issued and outstanding at September 30, 2025 and December 31, 2024.	—	—
Common stock, par value $0.0001; 125,000,000 shares authorized as of September 30, 2025 and December 31, 2024; 64,544,096 and 31,574,657 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively.	6	3
Additional paid-in capital	178,027	145,719
Accumulated deficit	(172,066)	(138,998)
Total stockholders' equity	5,967	6,724
Total liabilities, Series A preferred stock and stockholders' equity	$ 36,122	$ 36,862

Opus Genetics, Inc.
Condensed Consolidated 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,	
	2025	**2024**	**2025**	**2024**
License and collaborations revenue	$ 3,079	$ 3,867	$ 10,331	$ 6,690
Operating expenses:				
General and administrative	4,981	2,894	17,093	10,918
Research and development	6,409	8,982	20,384	19,817
Total operating expenses	11,390	11,876	37,477	30,735
Loss from operations	(8,311)	(8,009)	(27,146)	(24,045)
Fair value change in warrant and other derivative liabilities	(9,525)	—	(5,803)	—
Financing costs	—	—	(1,337)	—
Interest expense	(68)	—	(68)	—
Other income, net	450	483	1,286	1,648
Loss before income taxes	(17,454)	(7,526)	(33,068)	(22,397)
Benefit (provision) for income taxes	—	—	—	—
Net loss	(17,454)	(7,526)	(33,068)	(22,397)

Other comprehensive loss, net of tax	—	—	—	—
Comprehensive loss	$ (17,454)	$ (7,526)	$ (33,068)	$ (22,397)
Net loss per share:				
Basic and diluted	$ (0.25)	$ (0.29)	$ (0.59)	$ (0.88)
Number of shares used in per share calculations:				
Basic and diluted	70,636,887	26,145,080	56,100,689	25,501,117

Source: Opus Genetics, Inc.

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