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Opus Genetics Announces Updates on OPGx-LCA5 Clinical Program

First patient dosed in the pediatric cohort of the Phase 1/2 trial of OPGx-LCA5; initial data on the cohort anticipated by Q3 2025
New 12-month data on the first three adult OPGx-LCA5 patients to be presented at a major medical conference in Q2 2025
FDA meeting scheduled in March 2025 to discuss Phase 3 trial design and registrational endpoints for OPGx-LCA5

DURHAM, N.C., Feb. 18, 2025 (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 therapies to treat other ophthalmic disorders, today announced that the first pediatric patient was dosed in its ongoing Phase 1/2 clinical trial evaluating OPGx-LCA5, its investigational gene therapy for the treatment of Leber congenital amaurosis (LCA). Opus plans to share initial data from the pediatric cohort by Q3 2025.

The ongoing trial has achieved early clinical proof of concept, demonstrating meaningful visual improvement starting as early as one month following treatment in the first three adult patients, as was evident in the 6-month data first released in October 2024 and reviewed at a company-sponsored KOL event in December 2024 (HERE). Opus now plans to share the new 12-month data on these three adult LCA5 patients at a major medical conference during the second quarter of 2025. An FDA Type D meeting is scheduled in March to discuss the pivotal trial design and endpoints.

“We are proud of the progress of our ongoing OPGx-LCA5 clinical trial and pleased to reach this critical next step as we expand the trial to pediatric patients. Early intervention in pediatric LCA5 patients is particularly important, as it offers the best chance to preserve or restore visual function before the disease progresses,” said George Magrath, M.D., Chief Executive Officer of Opus Genetics. “We are encouraged by the new 12-month results that confirm the durability of the positive response observed at 6 months. This data will be shared in our scheduled meeting with the FDA in March to discuss a proposed Phase 3 trial design and registrational endpoints for the OPGx-LCA5 program. We are hopeful that this may offer a potentially life-changing therapeutic option for individuals living with LCA and will continue to work closely with the medical and patient communities to advance this important program. We’re grateful to our partners at the University of Pennsylvania for their hard work and dedication to our program.”

Phase 1/2 Trial Design

This clinical trial was designed to evaluate the safety and preliminary efficacy of subretinal

gene therapy with OPGx-LCA5 in patients with inherited retinal degeneration due to biallelic mutations in the LCA5 gene. It is an open-label, Phase 1/2 trial evaluating OPGx-LCA5. Efficacy endpoints include measurement of functional vision using: 1) the Multi-Luminance orientation and Mobility Test (MLoMT); 2) Full-Field Stimulus Testing (FST), which measures the retina's sensitivity to light; and 3) microperimetry, which measures point-wise sensitivity to light. For more information, visit clinicaltrials.gov (NCT05616793).

The six-month results on adult patients treated with OPGx-LCA5 were presented in a Key Opinion Leader (KOL) webinar, hosted by Opus on December 11, 2024. A replay of the webinar can be accessed here.

About OPGx-LCA5

OPGx-LCA5 is designed to address a form of Leber congenital amaurosis (LCA) due to biallelic mutations in the LCA5 gene (LCA5), which encodes the lebercilin protein. LCA5-associated inherited retinal disease is an early-onset severe inherited retinal dystrophy. Studies in patients with this mutation have reported evidence for the dissociation of retinal architecture and visual function in this disease, suggesting an opportunity for therapeutic intervention through gene augmentation. OPGx-LCA5 uses an adeno-associated virus 8 (AAV8) vector to precisely deliver a functional LCA5 gene to the outer retina. OPGx-LCA5 is currently being evaluated in a Phase 1/2 clinical trial at the University of Pennsylvania designed to evaluate its safety and preliminary efficacy in patients with inherited retinal degeneration due to biallelic mutations in the LCA5 gene.

About Opus Genetics

Opus Genetics is a clinical-stage ophthalmic biotechnology company developing gene therapies to treat patients with IRDs and other treatments for ophthalmic disorders. The 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 trial, with encouraging early data. BEST1 investigational gene therapy is designed to address mutations in the BEST1 gene, which is associated with retinal degeneration; we expect that a Phase 1/2 trial to be initiated in 2025. 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 (DR). Phentolamine Ophthalmic Solution 0.75% is currently being evaluated in Phase 3 trials for treatment of presbyopia and reduced dim (mesopic) light vision following keratorefractive surgery. We have reached agreement with the FDA on a SPA for a Phase 3 trial to evaluate oral APX3330 for the treatment of DR. 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 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:

- Our ability to successfully integrate the business of former Opus Genetics Inc. and manage our expanded combined product pipeline;
- Our ability to develop and obtain regulatory approval for newly acquired gene therapies to treat inherited retinal diseases;
- Our ability to obtain and maintain orphan drug designation or rare pediatric disease designation for our current and future product candidates;
- 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;
- Rapid technological change;
- Our development of sales and marketing infrastructure;
- Future revenue losses and profitability;
- 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;
- Our dependency on key personnel;
- Changes in market opportunities and acceptance;
- Reliance on third parties to conduct our clinical trials and supply and manufacture drug supplies;
- 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 licensing or partnership arrangements may not facilitate the commercialization or market acceptance of our product candidates;
- Future fluctuations in the market price of our common stock;
- The success and timing of commercialization of any of our product candidates;
- Obtaining and maintaining our intellectual property rights; and
- The success of mergers and acquisitions.

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.

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