March 5, 2025

Opus Genetics Announces Presentations at Association for Research in Vision and Ophthalmology (ARVO) 2025 Meeting

New Data from First Three Adult Patients in Phase 1/2 Trial with OPGx-LCA5 Showed that Subjective and Objective Signs of Efficacy Persisted for One Year

Durham, NC., March 05, 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 other ophthalmologic disorders, today announced that three abstracts on its investigational gene therapy candidates have been accepted for presentation at the Association for Research in Vision and Ophthalmology (ARVO) 2025 Meeting, to take place May 4-8, 2025 in Salt Lake City, UT. The abstracts feature 12-month data from the first three adult patients in our ongoing Phase 1/2 trial of OPGx-LCA5, as well as pre-clinical results on OPGx-MERTK and OPGx-RDH12.

An abstract summarizing a subset analysis from the previously completed LYNX-1 Phase 3 trial of Phentolamine Ophthalmic Solution 75% has also been accepted for presentation. The full abstracts are available in the ARVO Online Planner, which can be accessed here.

“We are pleased to have the opportunity to share data on our gene therapy candidates and to engage with the global ophthalmology community at ARVO 2025,” said George Magrath, M.D., Chief Executive Officer at Opus Genetics. “We look forward to presenting the 12-month data on adult patients being treated in the ongoing Phase 1/2 trial of our most advanced gene therapy candidate OPGx-LCA5. Assuming continued safety and efficacy in the current study, we plan to advance OPGx-LCA5 into a pivotal Phase 3 trial and we are hopeful that, if successful in Phase 3, and approved, OPGx-LCA5 may offer a potentially life-changing therapeutic option for individuals living with LCA5.”

Abstract details

Title:	**Recovery of cone mediated vision in severe ciliopathy after gene augmentation; One year results from a Phase I/II trial of LCA5-LCA (OPGx-LCA5)**
Author:	Tomas Aleman, M.D., Schele Eye Institute, University of Pennsylvania *et al*
Presentation time:	May 4, 2025 from 4:30 PM to 4:45 PM MT
Location:	Room 255E

OPGx-LCA5 is an investigational gene therapy for the treatment of Leber congenital amaurosis (LCA). The candidate is being evaluated in an ongoing non-randomized single ascending Phase 1/2 dose escalation study. Previously announced results showed OPGx-LCA5 to be well tolerated, with all three adult patients showing visual improvement at six months. New data from the study, to be presented at ARVO, show that subjective and objective signs of efficacy persisted for a year.

Title:	**Evaluation of MERTK gene therapy in RCS rats following a single bilateral subretinal injection**
Author:	Mayur Choudhary, PhD., Opus Genetics *et al*
Presentation time:	May 8, 2025 from 11:45 AM to 1:30 PM MT
Posterboard Number:	5944 - A0009

OPGx-MERTK is a gene therapy being developed by Opus Genetics to treat patients impacted by MERTK-related retinitis pigmentosa (RP). Results from a pre-clinical study which evaluated OPGx-MERTK in a rat model of RP will be presented at ARVO.

Title:	**Evaluation of ocular tolerability of OPGx-RDH12 by subretinal delivery in cynomolgus primates**
Author:	Ash Jayagopal, PhD., Opus Genetics *et al*
Presentation time:	May 5, 2025 from 8:30 AM to 10:15 AM MT
Posterboard Number:	1621 - B0205

OPGx-RDH12 is a gene therapy being developed to treat Leber congenital amaurosis 13 (LCA13), a genetic retinal dystrophy caused by mutations in the RDH12 gene. This pre-clinical study was conducted to evaluate the tolerability of OPGx-RDH12 in primates. Results from this pre-clinical study will be presented at ARVO.

Title:	**LYNX-1 Phase 3 trial of the safety and efficacy of phentolamine ophthalmic solution for the treatment of reduced mesopic low contrast vision: A subset analysis of keratorefractive subjects**
Author:	Kostas Charizanis, PhD., Opus Genetics *et al*
Presentation time:	May 4, 2025 from 8:00 AM to 9:45 AM MT
Posterboard Number:	173 - A0289

LYNX-1 was a randomized, double-masked, placebo-controlled trial of the safety and efficacy of Phentolamine Ophthalmic Solution 0.75% in subjects with dim light disturbances (DLD) of various etiologies. Top-line results were announced in 2022. The results to be presented at ARVO are from a subset analysis of the 25 post-LASIK subjects in LYNX-1 who had reduced mesopic low contrast visual acuity (mLCVA) and photic complaints in order to inform patient population selection for ongoing and future Phase 3 studies.

About Opus Genetics

Opus Genetics is a clinical-stage ophthalmic biotechnology company developing gene therapies to treat patients with inherited retinal diseases (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, dose-escalation 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 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 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 treatment of presbyopia and reduced dim (mesopic) light low contrast vision following keratorefractive surgery. FDA Fast Track Designation has been granted for Phentolamine Ophthalmic Solution 0.75% as treatment of significant chronic night driving impairment in keratorefractive patients with reduced mesopic vision. 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, including from generic versions of our product candidates;
- 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;
- Actions by activist stockholders;
- 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.

Contacts

Corporate	Investor Relations
Nirav Jhaveri CFO ir@opusgtx.com	Corey Davis, Ph.D. LifeSci Advisors cdavis@lifesciadvisors.com

Source: Opus Genetics, Inc.