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Opus Genetics Announces Presentations on Inherited Retinal Disease Programs at Medical Conferences in May

RESEARCH TRIANGLE PARK, N.C., May 12, 2025 (GLOBE NEWSWIRE) -- Opus Genetics, Inc. (Nasdaq: IRD), a clinical-stage biotechnology company developing gene therapies for the treatment of inherited retinal diseases (IRDs) and other treatments for ophthalmic disorders, today announced presentations related to its IRD gene therapy programs at these upcoming scientific conferences in May.

Presentation Details

American Society of Gene & Cell Therapy (ASGCT) 28th Annual Meeting

Title:	Evaluation of MERTK gene therapy in RCS rats following a single bilateral subretinal injection
Date/ time:	May 13, 2025, from 6:00 to 7:00 pm ET
Presenter:	Mayur Choudhary Ph.D., Principal Scientist, Opus Genetics
Format:	Poster
Location:	Poster Hall I2, Ernest N. Morial Convention Center, New Orleans

Highlights

- Retinitis pigmentosa (RP), a group of inherited retinal dystrophies affecting one in 3,000 to one in 7,000 people, is characterized by disruption of rod photoreceptors, leading to rod-cone degeneration. Approximately 3% of RP cases are caused by mutations in the proto-oncogene tyrosine-protein kinase MER (MERTK) gene.
- In this rat model of retinal degeneration, subretinal administration of OPGx-MERTK (1E10 vg/eye) demonstrated effective preservation of both photoreceptors and retinal function.
- OPGx-MERTK (AAV2-VMD2-hMERTK) is an investigational gene therapy developed by Opus Genetics to treat patients with MERTK-related RP.
- This preclinical study evaluated the efficacy of OPGx-MERTK in a Royal College of Surgeons (RCS) rat model, which exhibit a RPE phagocytosis defect due to a natural mutation in MERTK, following a single bilateral subretinal injection.

American Ophthalmological Society (AOS) 2025 Annual Meeting

Title: Creation of Endpoints for the First Interventional Gene Therapy Clinical Study in BEST Disease using Animal Model and Natural History Data

Date/ time: May 15 – May 17, 2025

Presenter: George Magrath, M.D., Chief Executive Officer, Opus Genetics

Format: Poster

Location: The Ritz-Carlton Hotel, Naples, FL

Highlights

- OPGx-BEST1 is being developed for bestrophin-1 (BEST1)-related inherited retinal diseases or bestrophinopathies, a form of macular degeneration found primarily in adults.
- Results from a preclinical study evaluating the safety and efficacy of OPGx-BEST1 in a canine model of BEST1 related IRD will be presented.
- Restoration of the RPE-photoreceptor interface was demonstrated in the treated animals, including resolution of the retinal microdetachments.
- This study will inform endpoint selection in the first planned clinical study in patients with autosomal dominant and recessive BEST disease.
- Opus plans to commence a Phase 1/2 trial of OPGx-BEST1 in 2025 and aims to obtain preliminary data by Q1 2026.

Retinal Imaging Biomarkers and Endpoints Summit

Title: Utilizing VR Guided Multi Luminance Orientation and Mobility Testing for Assessment of Visual Function

Date/ time: Thursday, May 29, 2025 at 9:00 am ET

Presenter: Ash Jayagopal, PhD., Chief Scientific & Development Officer, Opus Genetics

Format: Oral Presentation

Location: Hilton Hotel, Boston MA

Highlights

- The presentation will explore how virtual reality stimulators can help connect structural changes in the retina with functional outcomes.
- Dr. Jayagopal will explain how these tests can be leveraged to help define clinically meaningful improvements.
- He will also discuss how secondary or exploratory functional endpoints can be validated and transitioned into primary endpoints for clinical trials.

About Opus Genetics

Opus Genetics is a clinical-stage ophthalmic biopharmaceutical company developing gene 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 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 dim (mesopic) light vision disturbances. For additional 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 Annual Report on Form 10-K for the fiscal year ended December 31, 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 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 markets;
- 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; 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
Nirav Jhaveri
CFO
ir@opusgtx.com

Investor Relations
Corey Davis, Ph.D.
LifeSci Advisors
cdavis@lifesciadvisors.com

Media
Kimberly Ha
KKH Advisors
kimberly.ha@kkhadvisors.com

Source: Opus Genetics, Inc.