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Opus Genetics Granted FDA Regenerative Medicine Advanced Therapy (RMAT) Designation for OPGx-LCA5 Gene Therapy Candidate

- FDA RMAT designation recognizes encouraging early data from Opus' Phase 1/2 trial in patients with LCA5, an ultra-rare inherited retinal disease

RESEARCH TRIANGLE PARK, N.C., May 06, 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 ophthalmic disorders, announced today that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to OPGx-LCA5, its investigational gene therapy for the treatment of Leber Congenital Amaurosis (LCA) due to genetic variations in the LCA5 gene.

The RMAT designation for OPGx-LCA5 is based on early clinical evidence from Opus's ongoing Phase 1/2 open-label, dose-escalation trial, which is evaluating the safety and potential efficacy of OPGx-LCA5 in patients with severe vision loss due to confirmed mutations in the LCA5 gene. The FDA's decision indicates recognition of the strength of this initial data and the unmet need in this patient population.

"The FDA's decision to grant RMAT designation to OPGx-LCA5 is a major milestone for the LCA5 patient community and a strong validation of our early clinical data," said George Magrath, M.D., Chief Executive Officer, Opus Genetics. "We're encouraged by the potential of OPGx-LCA5 to meaningfully impact patients living with this ultra-rare and debilitating form of inherited blindness, and we look forward to continued collaboration with the FDA to accelerate its development."

In addition to the RMAT designation, Opus has been invited to participate in the FDA's Initial Comprehensive Multidisciplinary RMAT Meeting to support Opus' development and manufacturing strategy. Lastly, Opus has been invited to join the FDA's Chemistry, Manufacturing and Controls (CMC) Development and Readiness Pilot (CDRP) program, which provides additional guidance for accelerating CMC development of products under an investigational new drug application.

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.

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 with Dr. Tomas Aleman 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 biopharmaceutical company developing 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 mutations in genes that cause different forms of bestrophinopathy, Leber congenital amaurosis (LCA) and retinitis pigmentosa. Our most advanced investigational gene therapy program, which has been granted the Regenerative Medicine Advanced Therapy (RMAT) designation by the FDA, is designed to address mutations in the LCA5 gene, which encodes the lebercilin protein. OPGx-LCA5 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, 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 presbyopia and dim (mesopic) light vision disturbances. We have reached agreement with the FDA under SPA for a Phase 3 trial to evaluate oral APX3330 for the treatment of DR 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 our interactions with the FDA, our participation in the CDRP program, and the effects of RMAT designation for OPGx-LCA5.

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:

- Clinical data referenced in this press release is preliminary and related to a relatively small group of patients, and, as a result, data that initially appears 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 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



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