February 26, 2025

Opus Genetics Announces FDA Fast Track and Enrollment Updates for Phentolamine Ophthalmic Solution 0.75% Programs

FDA Fast Track Designation granted for Phentolamine Ophthalmic Solution 0.75% as treatment of significant chronic night driving impairment in keratorefractive patients with reduced mesopic vision

Enrollment completion in LYNX-2 pivotal Phase 3 trial expected in first half of 2025

Enrollment now complete in VEGA-3 pivotal Phase 3 trial evaluating Phentolamine Ophthalmic Solution 0.75% for presbyopia

DURHAM, N.C., Feb. 26, 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, today announced completion of enrollment in the VEGA-3 Phase 3 clinical trial evaluating Phentolamine Ophthalmic Solution 0.75% for presbyopia. Opus also announced that enrollment in the LYNX-2 pivotal Phase 3 trial, evaluating Phentolamine Ophthalmic Solution 0.75% for the treatment of visual loss in low light conditions associated with keratorefractive surgery, is well-underway with anticipated completion of enrollment in the first half of 2025. In addition, the FDA has granted Fast Track designation for Phentolamine Ophthalmic Solution 0.75% as treatment of significant chronic night driving impairment with concomitant increased risk of motor vehicle accidents and debilitating loss of best spectacle corrected mesopic vision in keratorefractive patients with photic phenomena (i.e., glare, halos, starburst). Fast track status is designated to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need.

“We are pleased with the ongoing progress of both the VEGA-3 and LYNX-2 studies,” said Jay Pepose, M.D., PhD., Chief Medical Advisor at Opus Genetics. “Presbyopia affects millions of people and represents a daily challenge that can diminish independence and quality of life. Phentolamine Ophthalmic Solution 0.75% has the potential to transform how this condition is managed by offering a durable, non-invasive solution that improves near vision, without compromising nighttime distance vision. It also offers the potential to improve visual performance in patients who have undergone LASIK for vision correction, and who now have difficulty with low light vision and night-time vision disturbances. We look forward to sharing the results of these important studies and working with our partner to complete development in these two indications.”

VEGA-3 Phase 3 Program in Presbyopia

VEGA-3 is a randomized, double-masked, placebo-controlled, multi-center, Phase 3 clinical trial evaluating Phentolamine Ophthalmic Solution 0.75% in 545 participants with presbyopia. The primary endpoint is the percentage of participants with 15-letter improvement in photopic binocular distance-corrected near visual acuity ("DCNVA") on the eighth day following their first visit. Participants are being followed a total of 48 weeks to collect chronic safety data. Recruitment has taken place at 39 investigational sites in the U.S.

For more information on the VEGA-3 trial design and endpoints, please refer to www.ClinicalTrials.gov (NCT06542497).

LYNX-2 Phase 3 Program in Dim Light Disturbances

LYNX-2 is a randomized, double-masked, placebo-controlled Phase 3 clinical trial designed to evaluate Phentolamine Ophthalmic Solution 0.75% compared to placebo in subjects who underwent keratorefractive surgery and then reported decreased visual acuity under low light conditions. Target enrollment is 200 subjects, and the trial is more than 95% enrolled. The primary endpoint is a gain of 3 lines (or 15 letters) or more of distance vision improvement on a low contrast chart in low light conditions after 15 days of dosing.

The LYNX-2 trial is being conducted under conditions of a Special Protocol Assessment (SPA) with the U.S. FDA. Additional information about LYNX-2 can be found at www.clinicaltrials.gov NCT06349759**.**

The U.S. FDA recently granted Fast Track designation for Phentolamine Ophthalmic Solution 0.75% as treatment of significant chronic night driving impairment in keratorefractive patients with reduced mesopic vision and photic phenomena. Fast Track designation is an important regulatory milestone with the potential to accelerate the development and review of new drugs intended to treat serious conditions with unmet medical needs. This designation offers Opus Genetics several key benefits, including more frequent interactions with the FDA, eligibility for Priority Review, as well as rolling review, allowing sections of the NDA to be submitted and evaluated on an ongoing basis.

About Presbyopia

Presbyopia is the most common age-related ocular condition. It is estimated that 128 million Americans, and over 2 billion people worldwide, have presbyopia, and this number is expected to grow as the population ages. Presbyopia reduces the eye's ability to focus on near objects due to the loss of lens elasticity and its ability to change shape. This progressive condition typically affects individuals over the age of 40 and can significantly impact quality of life and ability to perform everyday tasks such as reading, using digital devices, and other close-up activities. Presbyopia leads to the widespread use of reading glasses or bifocals. Phentolamine Ophthalmic Solution 0.75% is being developed to provide a non-invasive, convenient alternative to traditional corrective measures.

About Decreased Vision in Low Light Conditions after Keratorefractive Surgery

Decreased low contrast visual acuity under low light conditions occurs when the pupil dilates in low light conditions allowing peripheral unfocused rays of light to enter the eye, degrading image quality. It is not correctable with glasses and is often accompanied by glare, halos and starbursts at night. The condition is common in patients with increased peripheral ocular

aberrations and ocular scatter from refractive surgery (including LASIK, PRK, SMILE, and RK). There are currently no FDA-approved treatments. Phentolamine Ophthalmic Solution 0.75% has a mechanism of action that moderately reduces pupil size, thereby blocking unfocused peripheral rays of light, without the increased risks of retinal tears or detachment associated with parasympathomimetic miotics that engage the ciliary muscle. It has the potential to be a treatment option that could improve patients' ability to see, drive and function in low light.

About Phentolamine Ophthalmic Solution 0.75%

Phentolamine Ophthalmic Solution 0.75%, Opus Genetics' late-stage product candidate, is a non-selective alpha-1 and alpha-2 adrenergic antagonist designed to reduce pupil size. It works by uniquely blocking the alpha-1 receptors found on the radial iris dilator muscles, which are activated by the alpha-1 adrenergic receptors, without affecting the ciliary muscle. Phentolamine Ophthalmic Solution 0.75% is being developed for presbyopia and reduced mesopic low contrast and night vision disturbances after keratorefractive surgery.

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 gene therapies that address mutations in genes that cause different forms of bestrophinopathy, Leber congenital amaurosis (LCA) and retinitis pigmentosa. The company's most advanced 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 gene therapy is designed to address mutations in the BEST1 gene, which is associated with retinal degeneration; 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 to reduce pupil size, and APX3330, a novel small-molecule inhibitor of Ref-1 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. 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 expectations regarding our cash runway, data from and future enrollment for our clinical trials, our pipeline of additional indications, expectations of potential growth, and our expectations regarding integration following the acquisition of Opus Genetics, including with respect to the combination of their portfolio of clinical assets into our existing portfolio and our combined focus on gene therapy treatment.

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 Ocuphire’s Annual Report on Form 10-K. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this report. 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:

- 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 and rapid technological change;
- Our development of sales and marketing infrastructure;
- Future revenue losses and profitability;
- Our relatively short operating history;
- 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;
- Employee misconduct;
- Reliance on third parties;
- 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 partnership or other licensing arrangements, may not facilitate the commercialization or market acceptance of our product candidates;
- Future fluctuations in the market price of our common stock;
- Our ability to realize the expected benefits of the acquisition of Opus Genetics;
- Our ability to execute clinical programs for gene therapies successfully and changes in

expected commercial value we predict from the development of gene therapies;

- The success and timing of commercialization of any of our product candidates; and
- Obtaining and maintaining our intellectual property rights.

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.