October 22, 2024

Ocuphire Pharma Announces Acquisition of Opus Genetics

Acquisition creates a leading, clinical-stage company focused on the development of gene therapy treatments for rare inherited retinal degenerations

New OPGx-LCA5 Phase 1/2 6-month data demonstrate safety and visual improvement in early onset retinal degeneration

Additional clinical data in LCA5 pediatric patients and BEST1 patients is expected in 2H 2025

LYNX-2 Phase 3 trial of Phentolamine Ophthalmic Solution 0.75% in patients with dim light disturbances remains on track for top-line data in Q1 2025

VEGA-3 Phase 3 trial of Phentolamine Ophthalmic Solution 0.75% in presbyopia remains on track for top-line data in H1 2025

Company will seek a strategic partner to continue development of APX3330, an oral small-molecule inhibitor of Ref-1 for the treatment of non-proliferative diabetic retinopathy

Projected cash runway extended into 2026

Conference call to discuss the acquisition to take place at 4:30 p.m. ET today

FARMINGTON HILLS, Mich., Oct. 22, 2024 (GLOBE NEWSWIRE) -- Ocuphire Pharma, Inc. (Nasdaq: OCUP), a clinical-stage ophthalmic biopharmaceutical company focused on developing and commercializing therapies for the treatment of patients with retinal and refractive eye disorders, today announced the all-stock acquisition of Opus Genetics, Inc., a clinical-stage gene therapy company for inherited retinal diseases (IRDs). The merger creates a transformative biotech company committed to being a leader in the development of gene therapies for the treatment of IRDs. In connection with the merger, the combined company will be renamed Opus Genetics, Inc., effective October 23, 2024, and will trade on Nasdaq under the ticker symbol “IRD” effective October 24, 2024.

“Opus Genetics has created a compelling pipeline of transformative therapies for patients with inherited retinal diseases, with promising early data. This is an opportunity to advance these treatments quickly, with four major clinical milestones on the horizon in 2025 for the combined company,” said George Magrath, M.D., who will continue to serve as CEO of the combined company. “We are encouraged by the new LCA5 six-month proof-of-concept data showing visual improvement in three out of three patients with advanced disease and are excited to bring together a leadership team with deep expertise in the development of potentially groundbreaking gene therapies. We look forward to continuing our progress,

creating value, and improving patient outcomes together."

Ben Yerxa, Ph.D., former President and CEO of Opus Genetics and President of the newly combined company, added, "With the Ocuphire team's late-stage ophthalmic drug development and regulatory approval experience and resources, we believe we are well-positioned to accelerate our pipeline of potentially transformative gene therapies for inherited retinal diseases. We see this transaction as a win for patients with IRDs around the world, and we look forward to efficiently progressing our combined pipeline."

The combined company now has an expanded pipeline that includes multiple compelling assets from its adeno-associated virus (AAV)-based gene therapy portfolio, which is currently being developed for IRDs, as well as Phentolamine Ophthalmic Solution 0.75%, which is currently being evaluated in presbyopia and dim (mesopic) light vision disturbances (sometimes referred to as DLD) after keratorefractive surgery. Due to the capital requirements and developmental timelines of APX3330, an oral small-molecule inhibitor of Ref-1 for the treatment of non-proliferative diabetic retinopathy, the company will seek a strategic partner to advance the clinical development of the late-stage diabetic retinopathy program and will redirect its existing resources towards the acquired gene therapy programs.

The most advanced gene therapy candidate, OPGx-LCA5, is being developed to treat LCA5, an early-onset retinal degeneration, and an open-label, dose-escalation Phase 1/2 clinical trial is ongoing. The trial has shown early clinical proof-of-concept, with new six-month data demonstrating visual improvement in three out of three adult patients participating in the trial, each of whom has late-stage disease.

Jean Bennett, M.D., Ph.D., scientific co-founder of Opus Genetics, commented, "This level of efficacy in patients with late-stage disease is exciting and supportive of the potential for a one-time treatment with OPGx-LCA5, which could have a transformative impact on individuals who have experienced devastating vision loss and for whom no alternative treatment options exist."

Enrollment of the first pediatric patients in the Phase 1/2 trial is expected in the first quarter of 2025, with the first data anticipated in the third quarter of 2025. As the program has received Rare Pediatric Disease Designation and Orphan Drug Designation from the U.S. Food and Drug Administration (FDA), OPGx-LCA5 will be eligible to receive a priority review voucher upon biologics license application (BLA) approval.

Dr. Bennett, Dr. Yerxa and Adrienne Graves, Ph.D., each of whom has served on the Board of Directors of Opus Genetics prior to the transaction, will join the Board of Directors of the combined company. Dr. Bennett is the scientific co-founder of Opus Genetics and former scientific founder of Spark Therapeutics. She was one of the first investigators to use viral vectors to deliver transgenes to specific cells in the retina and led the first team to demonstrate proof-of-principle of ocular gene therapy. Dr. Yerxa co-founded Opus Genetics and was the former CEO of the Foundation Fighting Blindness and oversaw the establishment of the Foundation Fighting Blindness' Retinal Degeneration (RD) Fund. He has more than 30 years' experience in biotechnology and ophthalmic drug development, translating promising research discoveries into clinical milestones and treatments. Dr. Graves is the former CEO of Santen Inc., and former chair of Iveric Bio, and currently serves as board chair for the RD Fund.

The expected cash runway of the combined company has been extended into 2026, during which period the company anticipates clinical data readouts for pediatric patients in the OPGx-LCA5 Phase 1/2 trial, the initial patients of the OPGx-BEST1 Phase 1/2 trial, the LYNX-2 Phase 3 trial, and the VEGA-3 Phase 3 trial. The LYNX-2 Phase 3 trial in patients with decreased visual acuity under low light conditions following keratorefractive surgery and the VEGA-3 Phase 3 trial for presbyopia are actively enrolling, with top-line data expected in the first quarter and first half of 2025, respectively.

Terms of the Acquisition

In connection with the acquisition, Ocuphire issued 5.2 million shares of its common stock and 14.1 thousand shares of its convertible preferred stock to existing stockholders of Opus Genetics. The shares of convertible preferred stock will be convertible into shares of common stock, subject to stockholder approval at the company's annual meeting of stockholders, to be held in April 2025. Following the issuances, pre-acquisition stockholders of Ocuphire will own approximately 58% of the combined company's fully diluted capitalization, and pre-acquisition stockholders of Opus Genetics will own approximately 42% of the combined company's fully diluted capitalization.

Leerink Partners is serving as exclusive financial advisor to Ocuphire. Sidley Austin LLP is serving as legal counsel to Ocuphire. Smith Anderson is serving as legal counsel to Opus Genetics.

Investor Conference Call

Management will host a conference call and webcast with slides at 4:30 p.m. ET today to discuss the acquisition. Dial-in details are as follows:

Investors dial	1-877-407-0792
International investors dial	1-201-689-8263
Conference ID	13749219

Call me™: Participants can use Guest dial-in #s above and be answered by an operator OR click the Call me™ link for instant telephone access to the event. The Call me™ link will be made active 15 minutes prior to scheduled start time. CALL ME LINK

Webcast link: CLICK HERE

An archive of the call will be available on the company's corporate website for 90 days following the call.

About the Company

The company is a clinical-stage ophthalmic biopharmaceutical company developing therapies to treat patients with inherited retinal diseases (IRDs) and therapies to treat patients with other retinal and refractive 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. 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 presbyopia and dim (mesopic) light vision disturbances.

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 its product candidates and its preclinical programs;
- Domestic and worldwide legislative, regulatory, political and economic developments;
- Employee misconduct;
- Changes in market opportunities and acceptance;
- 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

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Source: Ocuphire Pharma; Opus Genetics