Ocular Therapeutix™ Announces 12-Month Topline Data from Ongoing U.S.-Based Phase 1 Clinical Trial Evaluating OTX-TKI for Treatment of Wet AMD

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OTX-TKI maintained vision and CSFT comparable to aflibercept every eight weeks, with 89% reduction in treatment burden over a 12-month period

OTX-TKI demonstrated no drug-related ocular or systemic serious adverse events in the trial through Month 12

Implant observed to bioresorb consistently at about eight to nine months in trial, informing potential redosing timeline

Investor/analyst conference call to discuss results to be held on Monday, June 12, at 8:30 a.m. ET

BEDFORD, Mass., June 10, 2023 (GLOBE NEWSWIRE) -- Ocular Therapeutix, Inc. (NASDAQ: OCUL), a biopharmaceutical company focused on the formulation, development and commercialization of innovative therapies for diseases and conditions of the eye, today announced 12-month data from its Phase 1 U.S. clinical trial evaluating OTX-TKI, the Company's axitinib intravitreal hydrogel implant, for the treatment of wet age-related macular degeneration (wet AMD). OTX-TKI is also being developed for the treatment of diabetic retinopathy and other retinal diseases.

The data are being presented by Arshad M. Khanani, MD, MA, principal investigator on the trial and Director of Clinical Research at Sierra Eye Associates today at 6:29 p.m. ET (4:29 p.m. MT) at the Clinical Trials at the Summit 2023 Meeting in Park City, Utah. The presentation can be accessed by visiting the scientific and medical presentations tab of the investor section of the Company's website at www.ocutx.com.

An investigational, bioresorbable hydrogel intravitreal implant, OTX-TKI is designed to continuously deliver a potent tyrosine kinase inhibitor, axitinib, for the treatment of wet AMD and other VEGF-mediated retinal diseases. The 12-month data demonstrated maintenance of controlled wet AMD subjects comparable to aflibercept injections every eight weeks with a single administration of OTX-TKI. Four subjects received rescue therapy for the first time at Month 12, indicating the waning of OTX-TKI's therapeutic effect and potential disease reactivation, which helps establish a re-dosing timeline for patients. No serious drug-related ocular or systemic adverse events have been observed in the trial.

"We are very pleased to see the durability in the 12-month OTX-TKI data in previously treated patients with wet AMD," said Antony Mattessich, President and Chief Executive Officer of Ocular Therapeutix. "Pharmacodynamic effects observed in this trial support the characteristics of a treatment for wet AMD with durability between 9 to 12 months with a single injection. Following discussions with the FDA, we have two potential pivotal designs and are prepared to initiate a first pivotal trial for OTX-TKI in wet AMD as early as the third quarter of this year. We are excited for what these data could mean for patients living with VEGF-mediated retinal diseases."

Summary of 12-Month Topline Data from U.S. Phase 1 Clinical Trial Evaluating OTX-TKI for Treatment of Wet AMD

The U.S.-based Phase 1 clinical trial is a prospective, multi-center, randomized, controlled study in subjects previously treated with anti-VEGF therapy that is evaluating a 600 µg dose of OTX-TKI in a single implant, with a 2 mg aflibercept injection four weeks after the implant, compared to 2 mg aflibercept injections administered every 8 weeks. The trial is designed to assess the safety, durability and tolerability of OTX-TKI, and to assess biological activity in subjects by measuring best corrected visual acuity (BCVA) and central subfield thickness (CSFT) of the retina.

The clinical trial enrolled a total of 21 subjects at six clinical sites in the United States, who were randomized 3:1 to an arm receiving a single OTX-TKI implant, with a 2 mg aflibercept injection four weeks after implant injection, and an arm receiving aflibercept injections every 8 weeks. One subject in the OTX-TKI arm was not treated per protocol and has been removed from the efficacy analysis, as the subject incorrectly received aflibercept instead of a sham injection at Month 3 and 5 visits.

As of the data cutoff of April 14, 2023, there were no drug-related ocular or systemic serious adverse events (SAEs) observed in the OTX-TKI arm. As the Company previously announced at the 10-month data readout, one SAE of endophthalmitis was observed in the OTK-TKI arm, which occurred following the mandated aflibercept injection at Month 1 and was assessed by the investigator as related to the injection procedure. There were no retinal detachment, retinal vasculitis, or implant migration into the anterior chamber adverse events observed in the OTX-TKI arm, and no subjects had dropped out of either arm as of the data cutoff.

The results showed subjects treated with a single OTX-TKI implant continued to demonstrate sustained BCVA (mean change from baseline of -1.0 letters) and CSFT (mean change from baseline of +20.2 µm) in the OTX-TKI arm at 12 months, which was comparable with the aflibercept arm (mean change from BCVA baseline of +2.0 letters; mean change from CSFT baseline of -2.2 µm). 60% of OTX-TKI subjects were rescue-free up to Month 12. At the Month 12 visit, an additional four of the subjects were rescued. Overall, an 89% reduction in treatment burden was observed in OTX-TKI treated subjects at 12 months.

"We are encouraged by the 12-month data as the performance of the OTX-TKI drug product continued to demonstrate that the implant bioresorbs at an average of about eight to nine months with axitinib remaining longer in the eye, potentially providing a window to re-dose patients before disease reactivation starts," said Rabia Gurses Ozden, MD, Chief Medical Officer of Ocular Therapeutix.

Dr. Khanani said: "We saw an 89% overall reduction in treatment burden in patients treated with OTX-TKI. Approximately 73% of patients either required no or only one supplemental anti-VEGF injection through Month 12, which is clinically meaningful and highlights the possibility of OTX-TKI being widely adopted in the management of wet AMD."

The Company is prepared to initiate a pivotal trial in wet AMD as early as the third quarter of 2023, subject to obtaining the necessary financing, which could be provided through a strategic alliance. OTX-TKI is also being evaluated in other retinal diseases. The Company recently completed enrollment of the Phase 1 HELIOS clinical trial to evaluate OTX-TKI for the treatment of diabetic retinopathy and 6-month interim results are expected in the first

quarter of 2024.

Conference Call & Webcast Information

Members of the Ocular Therapeutix management team, including Chief Medical Advisor, Retina, Peter K. Kaiser, MD, along with OTX-TKI U.S. Phase 1 clinical trial investigator, Dr. Khanani, will host a live conference call and webcast Monday, June 12, at 8:30 a.m. EDT for investors and analysts. Listeners can register for the webcast via this link. Analysts wishing to participate in the question and answer session should use this link. A replay of the webcast will be available via the investor section of the Company's website at www.ocutx.com approximately two hours after the call's conclusion for 90 days following the webcast. Those who plan on participating are advised to join 15 minutes prior to the start time.

About OTX-TKI

OTX-TKI is an investigational bioresorbable, hydrogel implant incorporating axitinib, a small molecule, multi-target, tyrosine kinase inhibitor with anti-angiogenic properties, being evaluated for the treatment of wet age-related macular degeneration (wet AMD) and other retinal diseases.

About Wet Age-Related Macular Degeneration

Wet age-related macular degeneration (wet AMD) is a leading cause of severe, irreversible vision loss affecting approximately 14 million individuals globally and 1.6 million in the United States alone (2022 Market Scope® Retinal Pharmaceuticals Market Report). Wet AMD causes vision loss due to abnormal new blood vessel growth and hyperpermeability and associated retinal vascularity in the macula, which is primarily stimulated by local upregulation of vascular endothelial growth factor (VEGF). Without prompt and continuous treatment to control this exudative activity, patients develop irreversible vision loss. With proper treatment, patients may maintain visual function for a period of time and may temporarily regain lost vision. Challenges with current therapies include repeated intraocular injections every 1 to 4 months, treatment-related adverse events, patient compliance, and lack of vision improvement.

About Ocular Therapeutix, Inc.

Ocular Therapeutix, Inc. is a biopharmaceutical company focused on the formulation, development, and commercialization of innovative therapies for diseases and conditions of the eye using its proprietary bioresorbable hydrogel-based formulation technology. Ocular Therapeutix's first commercial drug product, DEXTENZA®, is an FDA-approved corticosteroid for the treatment of ocular inflammation and pain following ophthalmic surgery and ocular itching associated with allergic conjunctivitis. Ocular Therapeutix's earlier stage development assets include: OTX-TKI (axitinib intravitreal implant), currently in Phase 1 clinical trials for the treatment of wet AMD and diabetic retinopathy; OTX-TIC (travoprost intracameral implant), currently in a Phase 2 clinical trial for the treatment of primary open-angle glaucoma or ocular hypertension; and OTX-CSI (cyclosporine intracanalicular insert) for the chronic treatment of dry eye disease and OTX-DED (dexamethasone intracanalicular insert) for the short-term treatment of the signs and symptoms of dry eye disease, both of which have completed Phase 2 clinical trials.

Forward Looking Statements

Any statements in this press release about future expectations, plans, and prospects for the Company, including the commercialization of DEXTENZA® or any of the Company's products or product candidates; the development and regulatory status of the Company's product candidates, such as the Company's development of and the timing of planned pivotal clinical trials for OTX-TKI for the treatment of retinal diseases including wet AMD and diabetic retinopathy; the Company's plans to advance the development of OTX-TKI; the ongoing development of the Company's extended-delivery hydrogel depot technology; the potential utility of any of the Company's product candidates; the size of the potential market for OTX-TKI; the Company's ability to fund the planned and future clinical development of its product candidates whether through strategic alliances or other fundraising; the Company's ability to enter into and perform its obligations under collaborations; the sufficiency of the Company's cash resources; and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend", "goal," "may", "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors. Such forward-looking statements involve substantial risks and uncertainties that could cause the Company's preclinical and clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the timing and costs involved in commercializing DEXTENZA or any product or product candidate that receives regulatory approval, including the conduct of post-approval studies, the ability to retain regulatory approval of DEXTENZA or any product or product candidate that receives regulatory approval, the ability to maintain and the sufficiency of product, procedure and any other reimbursement codes for DEXTENZA, the initiation, timing, conduct and outcomes of clinical trials, whether interim clinical trial data such as the data reported in this release will be indicative of the results of the trial upon its completion or subsequent clinical trials in this and other indications, availability of data from clinical trials and expectations for regulatory submissions and approvals, the Company's scientific approach and general development progress, the sufficiency of cash resources, the Company's existing indebtedness, the ability of the Company's creditors to accelerate the maturity of such indebtedness upon the occurrence of certain events of default, the Company's ability to enter into strategic alliances or generate additional funding on a timely basis, on favorable terms, or at all, the Company's ability to recruit and retain key personnel, and other factors discussed in the "Risk Factors" section contained in the Company's quarterly and annual reports on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views as of the date of this press release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so, whether as a result of new information, future events or otherwise, except as required by law. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this press release.

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