Ocular Therapeutix™ Announces Interim 10-month Data from the Ongoing U.S. Phase 1 Clinical Trial Evaluating OTX-TKI for the Treatment of Wet AMD

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All OTX-TKI treated subjects who were rescue-free at the Month 7 interim analysis remained rescue-free, extending the 73% rescue-free rate up to Month 10

CSFT and BCVA measurements at 10 months were comparable between OTX-TKI treated subjects and aflibercept treated subjects

OTX-TKI continued to be generally well tolerated with no drug-related serious adverse events through Month 10

Conference call to discuss results to be held on Monday, February 13 at 8:30 a.m. ET

BEDFORD, Mass., Feb. 11, 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 interim 10-month data from its U.S. Phase 1 clinical trial evaluating OTX-TKI, the Company's axitinib intravitreal hydrogel implant being developed for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy and other retinal diseases. The data is being presented this morning at 8:00 am ET at the Angiogenesis, Exudation, and Degeneration 2023 Virtual Meeting by Andrew A. Moshfeghi, MD, MBA. The presentation can be accessed by visiting the scientific and medical presentations tab of the investor section of the Company's website at investors.ocutx.com.

"We are very pleased to see this latest 10-month data that support OTX-TKI's potential to set a new standard for durability in the treatment of wet AMD," said Antony Mattessich, President and Chief Executive Officer. "We have a product candidate designed to continuously deliver axitinib, a TKI with the highest binding affinity for the VEGF receptor currently in clinical development for the treatment of wet AMD and other VEGF-mediated retinal diseases. These results demonstrated maintenance of controlled wet AMD subjects for up to 10 months with a single administration of OTX-TKI. We intend to discuss with the FDA future clinical trial requirements and will plan to initiate a first pivotal trial for OTX-TKI in the third quarter of this year, subject to financing including a potential strategic alliance. We are very excited for what these data could mean for patients suffering from any VEGF-mediated retinal disease – such as wet AMD, diabetic macular edema or diabetic retinopathy."

Summary of Interim 10-month Data from the Ongoing U.S. Phase 1 Clinical Trial Evaluating OTX-TKI for the Treatment of Wet AMD

The ongoing 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 U.S., who were randomized 3:1 to an arm receiving a single OTX-TKI implant, with a 2 mg aflibercept injection after implant, 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 December 12, 2022, in the U.S.-based Phase 1 clinical trial, the single OTX-TKI implant continued to be generally well tolerated with no drug-related ocular or systemic serious adverse events (SAEs) observed through 10 months. As the Company previously announced at the 7-month 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 elevated IOP, 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 interim results showed subjects treated with a single OTX-TKI implant demonstrated stable and sustained BCVA (mean change from baseline of -0.3 letters) and CSFT (mean change from baseline of -1.3 µm) in the OTX-TKI arm at 10 months, which was comparable with the aflibercept arm (mean change from BCVA baseline of -0.8 letters; mean change from CSFT baseline of -4.5 µm). The 73% of subjects who were rescue-free up to Month 7, continued to demonstrate OTX-TKI's extended duration of action and remained rescue-free up to Month 10. Overall, a 92% reduction in treatment burden was observed in OTX-TKI treated subjects for up to 10 months.

"We are very encouraged by the interim 10-month data being presented that builds upon the interim 7-month data shared last year

at the American Academy of Ophthalmology 2022 Annual Meeting," said Rabia Gurses-Ozden, MD, Chief Medical Officer. "OTX-TKI has been observed to generally be well tolerated with no serious drug-related ocular or systemic adverse events and the visual acuity and retinal thickness outcomes of subjects receiving OTX-TKI in the Phase 1 clinical trial have been comparable to those of subjects receiving aflibercept, while having a substantial reduction in treatment burden."

"Efficacious treatment options are available for our patients with wet AMD, but there is a big unmet need for a treatment that can be effective for more than 3 to 4 months with a single injection. The reduction in treatment burden seen over the first 10 months in patients in the OTX-TKI U.S. Phase 1 clinical trial who previously required many injections is very exciting," said Arshad M. Khanani, MD, MA, Director of Clinical Research at Sierra Eye Associates. "The data from the current trial is promising as 73% of the treated subjects were rescue-free up to Month 10. A drug with this durability would open up new opportunities in the treatment of retinal diseases."

The Company plans to meet with the FDA to discuss potential future clinical trial requirements. Subject to those discussions and obtaining the necessary financing, which could be provided through a strategic alliance, the Company intends to initiate a pivotal trial in wet AMD in the third quarter of 2023. Per protocol, the Company will continue to follow subjects in the Phase 1 trial at least until their respective one-year anniversaries of initial dosing. Given the potential broad applicability of OTX-TKI to other retinal diseases, the Company initiated a U.S.-based Phase 1 clinical trial to evaluate OTX-TKI for the treatment of diabetic retinopathy in December 2022.

Conference Call & Webcast Information

Members of the Ocular Therapeutix management team along with Chief Medical Advisor, Retina, Peter K. Kaiser, MD will host a live conference call and webcast Monday, February 13 at 8:30 am Eastern Time. 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 investors.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 2 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 1-4 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 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 severity and duration of the COVID-19 pandemic including its effect on the Company's revenues and relevant regulatory authorities' operations, 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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