



Ocular Therapeutix™ Announces Interim 7-month Data from U.S. Phase 1 Clinical Trial of OTX-TKI for the Treatment of Wet AMD

September 27, 2022

OTX-TKI was Well Tolerated and Demonstrated a Favorable Safety Profile with no Drug-Related Ocular or Systemic Serious Adverse Events (SAEs)

Sustained and Comparable CSFT and BCVA Measurements at 7 Months between OTX-TKI Treated Subjects and Aflibercept Treated Subjects

80% of Subjects were Rescue-Free up to 6 months and 73% of Subjects Were Rescue-Free up to 7 months Following a Single OTX-TKI Implant Injection

Conference Call to Discuss Results to be Held at 8:00 a.m. ET

BEDFORD, Mass--(BUSINESS WIRE)--Sep. 27, 2022-- 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 7-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) and other retinal diseases. The data will be presented in more detail at the upcoming American Academy of Ophthalmology (AAO) 2022 Annual Meeting Subspecialty Day being held in Chicago by Dilsher Dhoot, MD on Friday, September 30th at 3:29 CT and can be accessed approximately two hours after the presentation's conclusion for 90 days by visiting the investors section of the Company's website at investors.ocutx.com.

"These interim results from our U.S.-based Phase 1 trial of OTX-TKI for the treatment of wet AMD represent a significant milestone for Ocular," said Antony Mattessich, President and Chief Executive Officer. "Wet AMD is a leading cause of blindness affecting approximately 14 million individuals globally and approximately 2 million in the U.S. alone. The data presented today highlights the potential of OTX-TKI to become a highly-differentiated product capable of providing a durable anti-VEGF response that improves upon today's standard of care in the management of wet AMD and with potential in other retinal diseases. We look forward to discussing our future development plans with the FDA and, subject to those discussions, plan to initiate a Phase 2 clinical trial in the third quarter of 2023."

The U.S.-based Phase 1 clinical trial is a prospective, multi-center, randomized, controlled study 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 in subjects previously treated with anti-VEGF therapy. The trial is designed to assess the safety, durability and tolerability of OTX-TKI, and to assess biological activity in subjects by measuring visual acuity and anatomical changes of the retina using optical coherence tomography. The clinical trial enrolled a total of 21 subjects at six clinical sites in the U.S., who were randomized 3:1 to the OTX-TKI arm or the on-label every 8-week aflibercept injection arm.

Based on the data cutoff of August 24, 2022, interim data from the U.S.-based Phase 1 clinical trial showed that the single OTX-TKI implant was generally well tolerated with a favorable safety profile. There were no drug-related ocular or systemic serious adverse events (SAEs) observed. One SAE of endophthalmitis was observed in the OTX-TKI arm which occurred following the mandated aflibercept injection at Month 1 and assessed by the investigator as related to the injection procedure. There were no reported adverse events such as elevated IOP, retinal detachment, retinal vasculitis, or implant migration into the anterior chamber observed in the OTX-TKI arm, and no subjects dropped out of either arm.

There was one subject randomized to the OTX-TKI arm who was incorrectly given aflibercept instead of sham injections at the subject's Month 3 and Month 5 visits. These were not rescue treatments as the subject did not meet any rescue criteria. Since this subject was not treated according to protocol, the subject has been excluded from the analysis of biological activity (OTX-TKI: n=15 and Aflibercept: n=5) but included in the safety analysis (OTX-TKI: n=16 and Aflibercept: n=5).

The interim results showed subjects treated with a single OTX-TKI implant demonstrated stable and sustained best corrected visual acuity (BCVA) (mean change from baseline of -1.3 letters) and central subfield thickness (CSFT) (mean change from baseline of +9.2 µm) in the OTX-TKI arm at 7 months, which was comparable with the aflibercept arm dosed every 8 weeks (mean change from BVCA baseline of -1 letter; mean change from CSFT baseline of +0.4 µm). The data also showed that 80% of subjects in the OTX-TKI arm were rescue-free up to 6 months and 73% of subjects in the OTX-TKI arm were rescue-free up to 7 months. (Table 1). These data have not been reviewed by FDA.

Table 1. Monthly Rescue-Free Rates with OTX-TKI (n=15)

Visits	Month 2	Month 3	Month 4	Month 5	Month 6	Month 7
Percentage of OTX-TKI subjects Rescue-Free up to each Visit	100%	100%	93%	93%	80%	73%

"We are very encouraged by these interim results from our U.S.-based Phase 1 clinical trial of OTX-TKI for the treatment of wet AMD. This adds to our knowledge of the safety and biological activity of a single OTX-TKI implant in a different population of wet AMD than the Australia-based Phase 1 clinical trial where we are studying subjects with uncontrolled subretinal or intraretinal fluid. We are looking forward to advancing the development of OTX-TKI in wet AMD and other retinal diseases," said Rabia Gurses Ozden, MD, Chief Medical Officer at Ocular Therapeutix.

"The favorable safety profile, stable and sustained visual acuity and retinal thickness outcomes, and clinically meaningful reduction in treatment burden in this double-masked, randomized clinical trial of subjects controlled previously on standard of care anti-VEGF injections is very exciting," said Peter K. Kaiser, MD, Chaney Family Endowed Chair in Ophthalmology Research and Professor of Ophthalmology at the Cleveland Clinic Lerner College of Medicine and Chief Medical Advisor, Retina at Ocular Therapeutix.

Based on the positive data, the Company plans to complete its analysis of the interim U.S. Phase 1 data, meet with the FDA to discuss potential future clinical trial requirements and, subject to those discussions, initiate a Phase 2 wet AMD clinical trial in the third quarter of 2023. The Company also plans to follow subjects in the Phase 1 trial at least until their respective one-year anniversaries of initial dosing, in accordance with the clinical trial protocol. Given the potential broad applicability of OTX-TKI to other retina diseases, the Company also plans to initiate a U.S.-based Phase 1 clinical trial to evaluate OTX-TKI for the treatment of diabetic retinopathy in the first quarter of 2023.

OTX-TKI is protected by a composition of matter patent, U.S. Patent Number 11,439,592, with an expiration date of March 2041. Additional U.S. and foreign patent applications are pending.

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 today at 8:00 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 other retinal diseases; 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®, ReSure® Sealant, or any of the Company's 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; 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 potential market for OTX-TKI; 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, ReSure Sealant or any product candidate that receives regulatory approval, including the conduct of post-approval studies, the ability to successfully develop and commercialize products for the ophthalmology office setting, the ability to retain regulatory approval of DEXTENZA, ReSure Sealant or any 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, any additional financing needs, 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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