Ocular Therapeutix™ Presents Preclinical Data on Pharmacokinetics, Efficacy and Tolerability of Sustained Release Intravitreal Tyrosine Kinase Inhibitor Hydrogel Depot (OTX-TKI) at the ARVO Annual Meeting

May 9, 2017

BEDFORD, Mass.--(BUSINESS WIRE)--May 9, 2017-- Ocular Therapeutix, Inc. (NASDAQ:OCUL), a biopharmaceutical company focused on the development, manufacturing and commercialization of innovative therapies for diseases and conditions of the eye, today presented data from preclinical studies evaluating the efficacy, tolerability and pharmacokinetics of its sustained release intravitreal tyrosine kinase inhibitor (TKI) depot (OTX-TKI) using the Company's proprietary bioresorbable hydrogel fiber technology at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in Baltimore, MD.

Tyrosine kinase inhibitors have shown promise in the treatment of wet age-related macular degeneration (AMD). However, attempts to administer topical or systemic TKIs for AMD have been limited by bioavailability and off-target effects.

In this study, the OTX-TKI investigational drug product was well-tolerated, and high tissue levels of TKI were maintained for up to 6 months in Dutch belted rabbits. For the first time, the ability to deliver an efficacious dose of TKI to the posterior segment of the eye for the treatment of VEGF-induced retinal leakage was demonstrated for an extended duration of up to six months.

"These preclinical studies of OTX-TKI have demonstrated the ability to deliver and maintain high therapeutic tissue levels of TKI in the vitreous humor, retina and choroid," said Jonathan H. Talamo, M.D., Chief Medical Officer of Ocular Therapeutix. "With these positive results from our preclinical studies, we are looking forward to beginning our Phase 1 trial of OTX-TKI later this year."

Four posters evaluating the OTX-TKI depot were presented at the ARVO Annual Meeting:

- ***"Pharmacokinetics of a 6 month Sustained Hydrogel Delivery System for Tyrosine Kinase Inhibitors in Dutch Belted Rabbits"*** - Posterboard #B0488, Abstract #1984-B0488

The pharmacokinetics over 6 months of sustained delivery from the OTX-TKI depot placed by intravitreal injection in Dutch belted rabbits were evaluated. OTX-TKI produced significant and increasing levels of TKI in the vitreous humor, retina and choroid at months 1 through 6 without clinical evidence of local toxicity. Plasma levels were below the level of quantification, suggesting minimal risk of systemic toxicity.

- ***"Tolerability of a 6 month Sustained Hydrogel Delivery System for Tyrosine Kinase Inhibitors in Dutch Belted Rabbits"*** - Posterboard #B0460, Abstract #1956-B0460

The tolerability of sustained delivery of OTX-TKI depot delivered via intravitreal injection through 6 months was evaluated in naïve Dutch belted rabbits (n=9). Serial clinical examinations showed that OTX-TKI was well-tolerated, with no sign of inflammatory response or other abnormalities, and showed minimal histopathologic changes at 1, 3 and 6 months.

- ***"Tolerability of a Shape Changing Intravitreal Hydrogel Depot"*** - Posterboard #B0484, Abstract #1980-B0484

The tolerability of an OTX-TKI depot delivered via intravitreal injection in Dutch belted rabbits through 6 months was investigated. Serial clinical examinations showed no sign of inflammatory response or abnormal chorioretinal morphology. At 1, 3 and 6 months, histopathologic analysis demonstrated minimal changes from baseline measurement in cornea, conjunctiva, sclera, choroid, retina, and vitreous humor, suggesting the hydrogel was well-tolerated in the Dutch belted model.

- ***"Efficacy of a 6 month Sustained Hydrogel Delivery System for Tyrosine Kinase Inhibitors in a VEGF*** **Induced Retinal Leakage Model"** - Posterboard #B0472, Abstract #1968-B0472

The efficacy over 6 months of sustained delivery of an OTX-TKI depot placed via intravitreal injection was investigated in a Dutch belted rabbit, vascular endothelial growth factor (VEGF) induced, retinal leakage model. Eyes were challenged with VEGF injection at 2, 3 and 6 months and were evaluated for leakage. OTX-TKI significantly suppressed leakage and showed minimal to no vascular leakage through 6 months. Blank control eyes showed high tortuosity and leakage at all time points. For the first time, this study demonstrated the ability to deliver an efficacious dose of TKIs to the posterior segment of the eye using the OTX-TKI delivery platform, and the potential to serve as an alternative to the once every 4-6 week injections which are the current standard of care.

About OTX-TKI (tyrosine kinase inhibitor)

OTX-TKI is a preformed, bioresorbable hydrogel fiber depot with anti-angiogenic properties delivered by intravitreal tyrosine kinase inhibitor injection. Currently OTX-TKI is under initial preclinical investigation as a novel extended release drug product candidate.

About Retinal Diseases

Retinal diseases such as age-related macular degeneration (AMD), retinal vein occlusion (RVO) and diabetic macular edema (DME) can be devastating to eyesight, causing central and/or peripheral vision loss. AMD is one of the most common retinal diseases, affecting more than 2 million Americans alone, and is the leading cause of blindness in the United States.

Current therapies for retinal diseases require monthly injections, which have shown to manage the progression of these diseases to prevent further vision loss and, in some cases, gain visual acuity. However, these frequent intravitreal injections can increase the risk of infection, retinal detachment, and/or hemorrhage.

About Ocular Therapeutix, Inc.

Ocular Therapeutix, Inc. is a biopharmaceutical company focused on the development, manufacturing and commercialization of innovative therapies for diseases and conditions of the eye using its proprietary hydrogel platform technology. Ocular Therapeutix's lead product candidate, DEXTENZA™ (dexamethasone insert) 0.4 mg for intracanalicular use has completed Phase 3 clinical development for the treatment of ocular pain and inflammation following ophthalmic surgery. The FDA has accepted the Company's NDA resubmission for DEXTENZA for the treatment of ocular pain following ophthalmic surgery and has established a PDUFA target action date of July 19, 2017. If approved, the Company intends to submit a supplement to its NDA for ocular inflammation. OTX-TP (travoprost insert) is in Phase 3 clinical development for glaucoma and ocular hypertension. Ocular Therapeutix is also evaluating injectable drug delivery depots for back-of-the-eye diseases. Ocular Therapeutix's first product, ReSure® Sealant, is FDA-approved to seal corneal incisions following cataract surgery.

Forward Looking Statements

Any statements in this press release about future expectations, plans and prospects for the Company including the development and regulatory status of the Company's product candidates, such as the Company's expectations and plans regarding regulatory submissions for and the timing and conduct of clinical trials of DEXTENZA™ for the treatment of post-surgical ocular inflammation and pain, including our expectations regarding the NDA filed with the FDA and the FDA's response to the resubmitted NDA, and the potential impact of the re-inspection of manufacturing operations, DEXTENZA for the treatment of allergic conjunctivitis, DEXTENZA for the treatment of dry eye disease and OTX-TP for the treatment of glaucoma and ocular hypertension, the ongoing development of the Company's sustained release hydrogel technology, the potential utility of any of the Company's product candidates, potential commercialization of the Company's product candidates, 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 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, those related to the timing and costs involved in commercializing ReSure® Sealant or any product candidate that receives regulatory approval, the initiation and conduct of clinical trials, availability of data from clinical trials and expectations for regulatory submissions and approvals, the Company's scientific approach and general development progress, the availability or commercial potential of the Company's product candidates, the sufficiency of cash resources and need for additional financing or other actions 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 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. 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 release.

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