Ocular Therapeutix™ Completes Enrollment of the HELIOS Clinical Trial of OTX-TKI for the Treatment of Diabetic Retinopathy

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Interim 6-month Data Expected in the First Quarter of 2024

BEDFORD, Mass., June 07, 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 completion of enrollment in the HELIOS clinical trial of OTX-TKI. Using Elutyx™ technology, OTX-TKI is the Company's axitinib intravitreal implant that is being developed for the treatment of diabetic retinopathy, wet AMD and other retinal diseases.

Diabetic retinopathy is a leading cause of vision loss in working-age adults, affecting approximately 140 million individuals around the world, including 8.4 million people in the United States alone. Diabetic retinopathy can lead to vision loss and even blindness due to high levels of blood sugar in the eyes that damage the blood vessels in the retina, the light-sensitive tissue at the back of the eye.

"Millions of people suffer from diabetic retinopathy. Today, the current standard of care in non-proliferative stages is primarily 'watchful waiting', as frequent anti-VEGF injections and office visits pose a significant treatment burden primarily for the working-age population. Many patients frequently progress to more severe vision-destroying disease before they seek treatment, making diabetic retinopathy one of the leading causes of blindness among working-age adults in the U.S.," said Rabia Gurses Ozden, MD, Chief Medical Officer of Ocular Therapeutix. "We believe the same attributes that make OTX-TKI a compelling product candidate for the treatment of wet AMD – the ease of use of an office-based injection and the potential for long-term durability that reduces the treatment burden and need for frequent, invasive injections – could establish OTX-TKI as the standard of care in the treatment of diabetic retinopathy."

The HELIOS Phase 1 trial is a multi-center, prospective, masked, 2:1 randomized, controlled U.S.-based trial in 21 subjects evaluating 600 µg OTX-TKI dosed in a single implant containing axitinib compared to a sham injection procedure. Interim 6-month data from the trial are expected in the first quarter of 2024. The Company has been in discussions with the FDA for the clinical development of OTX-TKI for the treatment of diabetic retinopathy and has a potential pivotal design that is consistent with guidance from the FDA. Subject to favorable interim data from the ongoing clinical trial and obtaining the necessary financing to fund the trial, the Company plans to be prepared to initiate a Phase 3 pivotal trial for diabetic retinopathy as early as Q1 2024. This is in addition to the Company's plan to be prepared for initiation of a pivotal program in wet AMD as early as Q3 of 2023, subject to obtaining the necessary financing.

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), diabetic retinopathy and other retinal diseases.

About Diabetic Retinopathy

Diabetic retinopathy is a chronic, progressive disease driven by elevated levels of blood glucose and depleted levels of oxygen causing damage to blood vessels in the retina. Diabetic retinopathy is among the most common microvascular complications of diabetes and is a leading cause of blindness globally. Diabetic retinopathy can take time to develop, and may progress from early, mild, non-proliferative diabetic retinopathy (NPDR) to moderate and then severe NPDR and eventually to proliferative diabetic retinopathy (PDR). As patients progress from mild to severe NPDR, the risk of developing proliferative disease increases. PDR is the most serious stage of the disease and develops when areas of the retina that are starved for nourishment and oxygen, triggering the proliferation of new blood vessels via secretions of vascular endothelial growth factor (VEGF). Diabetic macular edema (DME), an accumulation of fluid in the macula, can occur at any stage of diabetic retinopathy. The current standard of care for NPDR is watchful waiting, although damage to blood vessels in the retina is ongoing in the background, with anti-VEGFs used more commonly when the disease has progressed to the proliferative stage. Regular eye examinations are crucial for early detection and prevention of diabetic retinopathy.

There were an estimated 8.4 million cases of diabetic retinopathy in the United States in 2022 according to Market Scope, of which 3.3 million cases were moderate to severe non-proliferative, growing at an approximately 2% compound annual growth rate through 2027. Overall, there are an estimated 141.2 million cases of diabetic retinopathy globally, growing at a compound annual growth rate of 3%. Prevalence of diabetic retinopathy is expected to increase due to population growth and increasing incidence of diabetes throughout much of the world.

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-DED (dexamethasone intracanalicular insert) for the short-term treatment of the signs and symptoms of dry eye disease; and OTX-CSI (cyclosporine intracanalicular insert) for the chronic treatment 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, timing of, and prospects for approvability of OTX-TKI for the treatment of retinal diseases including wet AMD and diabetic retinopathy including the timing of planned pivotal clinical trials, OTX-TIC for the treatment of primary open-angle glaucoma or ocular hypertension, OTX-DED for the short-term treatment of the signs and symptoms of dry eye disease, and OTX-CSI for the chronic treatment of dry eye disease; the Company's plans to advance the development of its product candidates or preclinical programs; the Company's ability to fund the planned and future development of its product candidates, whether through strategic alliances or other fundraising; the potential utility of any of the Company's product candidates; the size of potential markets for the Company's product candidates; 2023 financial guidance, including estimated net product revenue; 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 clinical trial data will be indicative of the results of subsequent clinical trials in the same or other indications or that interim data will be indicative of the full data from a clinical trial, uncertainties as to the timing and availability of data from clinical trials and expectations for regulatory submissions and approvals, the Company's ability to enter into and perform its obligations under collaborations and the performance of its collaborators under such collaborations, the Company's scientific approach and general development progress, the availability or commercial potential of the Company's product candidates, the Company's ability to meet supply demands, the Company's ability to generate its projected net product revenue and in-market sales on the timeline expected, if at all, 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, 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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