Ocular Therapeutix™ Announces First Patient Dosed in U.S.-based Phase 1 Clinical Trial of OTX-TKI for the Treatment of Wet Age-Related Macular Degeneration

July 29, 2021

First Clinical Trial to Assess a Single OTX-TKI Implant Containing a 600ug Dose

BEDFORD, Mass.--(BUSINESS WIRE)--Jul. 29, 2021-- 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 that it has dosed the first patient in the United States Phase 1 clinical trial of OTX-TKI (axitinib intravitreal implant) for the treatment of wet age-related macular degeneration (wet AMD).

“We are very excited to have recently begun dosing patients in this U.S. trial,” said Michael Goldstein, MD, MBA, President, Ophthalmology and Chief Medical Officer of Ocular Therapeutix. “The start of this trial is significant as this will be the first clinical trial that will dose subjects with our single, higher dose, 600 µg implant. While current anti-VEGF treatments are effective, their durability is limited. OTX-TKI has the potential to be a new sustained release administration with 6 months or longer durability and a new mechanism of action for the treatment of patients with wet AMD and other retinal diseases. Based on early data from our Australian Phase 1 clinical trial, OTX-TKI has initially demonstrated acceptable tolerability, preliminary biological activity in some patients and durability for up to six months or longer in some cases.”

The Phase 1 clinical trial in the U.S. is a prospective, randomized, controlled, multi-center trial evaluating a single OTX-TKI implant containing a 600 µg dose of axitinib, compared with a 2 mg dose of aflibercept administered every eight weeks in subjects previously treated with anti-VEGF therapy. This trial is designed to assess the safety, tolerability, and biological activity of OTX-TKI for the treatment of wet AMD. The U.S.-based clinical trial of OTX-TKI is being conducted under an exploratory IND (eIND) application at five sites with a total of 20 randomized subjects: 15 subjects being treated with a single OTX-TKI implant containing 600 µg dose of axitinib in combination with an anti-VEGF induction injection, and 5 subjects being treated at eight-week intervals with a dose of aflibercept.

About Age-Related Macular Degeneration

AMD, a progressive retinal disease that is the leading cause of blindness in adults over the age of 60, is estimated to affect approximately 11 to 15 million people in the U.S. AMD affects the center portion of the retina, called the macula, which is responsible for central vision and color perception. There are two forms of AMD; the dry form, which affects between 85-90% of patients, and the wet form, an aggressive form of AMD which affects the remaining 10-15% of patients. In patients with wet AMD, abnormal blood vessels grow underneath and into the retina. These abnormal blood vessels leak fluid and blood into and beneath the retina, causing vision loss.

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. Ocular Therapeutix has received a target action date under the Prescription Drug User Fee Act, commonly known as PDUFA, of October 18, 2021, for a supplemental new drug application for DEXTENZA to include an additional indication for the treatment of ocular itching associated with allergic conjunctivitis. Ocular Therapeutix’s earlier stage development assets currently in Phase 1 clinical trials include OTX-TKI (axitinib intravitreal implant) for the treatment of wet AMD and other retinal diseases and OTX-TIC (travoprost intracameral implant) for the reduction of intraocular pressure in patients with primary open-angle glaucoma or ocular hypertension. Ocular Therapeutix is currently evaluating each of 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 in Phase 2 clinical trials. Also, in collaboration with Regeneron, OTX-AFS (aflibercept suprachoroidal injection) is in pre-clinical development as an extended-delivery formulation of aflibercept for the treatment of retinal diseases. Ocular Therapeutix's first product, ReSure® Sealant, is an FDA-approved device to prevent wound leaks in 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 commercialization of DEXTENZA®, ReSure® Sealant, or any of the Company’s product candidates; the commercial launch of, and effectiveness of reimbursement codes for, DEXTENZA; the conduct of post-approval studies of and compliance with related labeling requirements for DEXTENZA and ReSure Sealant; the development and regulatory status of the Company’s product candidates, such as the Company’s development of and prospects for approvability of DEXTENZA for additional indications including the PDUFA target action date scheduled for October 18, 2021, for allergic conjunctivitis, OTX-CSI for the chronic treatment of dry eye disease, OTX-DED for the short-term treatment of the signs and symptoms of dry eye disease, OTX-TIC for the treatment of primary open-angle glaucoma or ocular hypertension, OTX-TKI for the treatment of retinal diseases including wet AMD, and OTX-AFS as an extended-delivery formulation of the VEGF trap aflibercept for the treatment of retinal diseases including wet AMD; the ongoing development of the Company’s extended-delivery hydrogel depot technology; the size of potential markets for our product candidates; the potential utility of any of the Company’s product candidates; the potential benefits and future operation of the collaboration with Regeneron Pharmaceuticals and other Company collaborations, including any potential future costs or payments thereunder; projected net product revenue, in-market sales and other financial and operational metrics of DEXTENZA and ReSure Sealant; potential market sizes for indications targeted by the Company’s product candidates, if approved; the expected impact of the COVID-19 pandemic on the Company and its operations; 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 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, 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 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, the severity and duration of the COVID-19 pandemic including its effect on the Company's and relevant regulatory authorities' operations, any additional financing needs 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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