Ocular Therapeutix™ Announces Initiation of its First Pivotal Clinical Trial of OTX-TKI in Wet AMD

October 3, 2023

Expected to Enroll Approximately 300 Evaluable Subjects Primarily in the United States

BEDFORD, Mass., Oct. 03, 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 the initiation of its first pivotal clinical trial to evaluate OTX-TKI, the Company's axitinib intravitreal implant, for the treatment of wet age-related macular degeneration (wet AMD). OTX-TKI is also being developed for the treatment of diabetic retinopathy and other retinal diseases.

"We are thrilled to announce the initiation of our pivotal trial evaluating OTX-TKI for the treatment of wet AMD and look forward to working with clinical sites across the US," said Antony Mattessich, CEO of Ocular Therapeutix. "With the activation of our first clinical site in the US, we believe we are on target to enroll our first subject before year end. The trial is a crucial step forward for our clinical program as we make progress toward our goal of bringing a transformative new treatment that can truly make a difference for wet AMD to patients coping with vision loss."

Ocular Therapeutix has requested a Special Protocol Assessment (SPA) from the U.S. Food and Drug Administration (FDA) regarding the design of the trial, which is designed as a superiority trial that will enroll approximately 300 evaluable wet AMD subjects who are treatment naïve in the study eye. The trial is designed to be a multi-center, parallel-group trial that will be run primarily at U.S. sites with subjects randomized to one injection of aflibercept or one implant of OTX-TKI followed by as needed supplemental anti-VEGF treatment based on pre-specified criteria. The safety and efficacy of OTX-TKI will be assessed by measuring best corrected visual acuity (BCVA) and central subfield thickness (CSFT) at 36 weeks. While this institutional review board (IRB) approval has been received, the Company does not intend to enroll a subject before the SPA feedback is received from the FDA.

Arshad M. Khanani, MD, MA, FASRS, Director of Clinical Research at Sierra Eye Associates in Reno, Nevada, has agreed to be the principal investigator for the OTX-TKI pivotal Phase 3 trial. Dr. Khanani is an internationally renowned retina specialist with extensive experience in clinical trial design for the treatment of age-related macular degeneration. He has served as a principal investigator for numerous clinical trials evaluating novel treatments for wet AMD and other retinal diseases.

"Anti-VEGF agents have revolutionized the treatment of wet AMD, but numerous real-world patients experience vision loss due to their inability to adhere to the demanding treatment regimen involving frequent injections," said Dr. Khanani. "OTX-TKI has shown the potential to reduce treatment burden while maintaining vision and anatomy for patients with wet AMD. The OTX-TKI pivotal trial design is unique as it is based on the latest FDA guidance for wet AMD. As a field, we must continue to push for the approval of new therapies while making sure we appropriately manage our patients who participate in these clinical trials. I look forward to working with Ocular Therapeutix, the regulatory agency and the retina community to harmonize regulatory endpoints with clinical practice."

David Brown, MD, FACS, a renowned leader in the retina community, is the co-chairman of the medical leadership board at Retina Consultants of America (RCA) which has agreed to participate in the trial. Dr. Brown has pioneered research and treatment in retina, publishing more than 300 national meeting presentations, abstracts and scientific papers, including many of the primary papers that established the use of anti-VEGF agents for AMD, diabetic retinopathy and retinal vein occlusion.

"This pivotal trial is thoughtfully designed to satisfy the FDA's latest guidance in wet AMD, while also balancing the needs of subjects who enter it," said Dr. Brown. "The sites at RCA are excited to be a part of the OTX-TKI program as we continue our mission to develop better treatments for patients in the fight against retinal blindness."

The pivotal trial was initiated based on the previously announced positive 12-month top-line data from the Company's 21-subject U.S.-based Phase 1 trial of OTX-TKI for the treatment of wet AMD. Data from this trial were presented at the Clinical Trials at the Summit 2023 Meeting held in Park City, Utah in June 2023. In that trial, subjects treated with a single implant of OTX-TKI were observed to have an 89% reduction in treatment burden compared to subjects treated with aflibercept while maintaining visual acuity and retinal thickness through 12 months. Subjects in the OTX-TKI arm had mean changes from baseline of -1.0 letters in BCVA and +20.2 µm in CSFT at 12 months, comparable to mean changes from baseline for subjects in the aflibercept arm of +2.0 letters and -2.2 µm. 60% of OTX-TKI subjects remained rescue-free up to 12 months. As of the April 14, 2023 data cut-off date, no drug-related ocular or systemic serious adverse events through 12 months were observed in the OTX-TKI arm.

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 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 a Phase 1 clinical trial for the treatment of diabetic retinopathy and a pivotal Phase 3 trial for wet AMD; 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 development and regulatory

status of the Company's product candidates, including the timing and design of the Company's pivotal trials of OTX-TKI for the treatment of wet AMD; the Company's plans to advance the development of OTX-TKI; the Company's cash runway and 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, design, timing, conduct and outcomes of clinical trials, including the first pivotal trial of OTX-TKI for the treatment of wet AMD; uncertainties as to the response from the FDA regarding the SPA submission for OTX-TKI, including the risk that the FDA will not agree with the design of the first pivotal trial under the SPA; the risk that even if the FDA agrees with the design of the first pivotal trial under the SPA, the FDA will not agree that the data generated by the trial could support marketing approval; uncertainty as to whether the data from earlier clinical trials will be predictive of the data of later clinical trials, particularly later clinical trials that have a different design than the earlier trials; availability of data from clinical trials and expectations for regulatory submissions and approvals; the Company's scientific approach and general development progress; uncertainties inherent in estimating the Company's cash runway, future expenses and other financial results, including its ability to fund future operations, including clinical trials; Company's existing indebtedness and the ability of the Company's creditors to accelerate the maturity of such indebtedness upon the occurrence of certain events of default; the Company's ability to enter into strategic alliances or generate additional funding on a timely basis, on favorable terms, or at all; 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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