Ocular Therapeutix™ Announces FDA Agreement to Amend Special Protocol Assessment (SPA) for Pivotal Clinical Trial of AXPAXLI™ in Wet AMD

January 25, 2024

SPA amendment expands eligibility criteria for SOL clinical trial

Broadening of eligibility criteria anticipated to accelerate overall trial enrollment

BEDFORD, Mass., Jan. 25, 2024 (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 that the U.S. Food and Drug Administration (FDA) has agreed to a Special Protocol Assessment (SPA) Agreement Modification for the Company's pivotal Phase 3 SOL clinical trial of AXPAXLI™ (axitinib intravitreal implant, also known as OTX-TKI) for the treatment of wet age-related macular degeneration (wet AMD).

The SPA Agreement Modification enables the trial to include treatment-naïve wet AMD subjects with visual acuity of approximately 20/80 or better at the initial screening visit. After two aflibercept injections in the screening period, eligible participants would need to gain at least 10 ETDRS letters from the initial screening visit to Day 1 or achieve a visual acuity of approximately 20/20 or better at Day 1, in addition to satisfying other criteria, to qualify for enrollment in the trial. The SPA Agreement Modification also allows the pivotal trial to move forward, evaluating AXPAXLI with a single optimized implant with a drug load of 450 µg of a more soluble form of axitinib. This optimized configuration is expected to provide for a slightly increased daily release of the drug and is designed to improve synchronization of axitinib drug depletion with hydrogel bioresorption.

"With the changes reflected in the SPA Agreement Modification agreed to by the FDA, we believe we can accelerate the enrollment process for the SOL trial," said Antony Mattessich, CEO of Ocular Therapeutix. "We have worked closely with the FDA to ensure that the SOL trial meets the agency's updated guidance for wet AMD clinical trials, and we are eager to advance our engagement with clinical sites to enroll this trial under these new criteria."

About AXPAXLI
AXPAXLI 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 AMD and other retinal diseases.

About Wet AMD
Wet age-related macular degeneration (wet AMD) is a leading cause of severe, irreversible vision loss affecting approximately 14 million individuals globally and 1.6 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 to 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 ELUTYX™. 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: AXPAXLI (axitinib intravitreal implant), currently in a pivotal Phase 3 trial for wet AMD and a Phase 1 clinical trial for the treatment of diabetic retinopathy; PAXTRAVA ™ (travoprost intracameral implant, also known as OTX-TIC), 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, design, and enrollment of the Company's pivotal trials of AXPAXLI (also called OTX-TKI) for the treatment of wet AMD; the Company's plans to advance the development of AXPAXLI; 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; 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 SOL trial; the risk that the FDA will not agree with the Company's interpretation of the written agreement under the SPA; the risk that even though the FDA has agreed with the overall design of the SOL trial, the FDA may not agree that the data generated by the SOL trial supports potential 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 or utilize a different formulation 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.

Investors
Ocular Therapeutix
Donald Notman
Chief Financial Officer
dnotman@ocutx.com

or

ICR Westwicke
Chris Brinzey, 339-970-2843
Managing Director
chris.brinzey@westwicke.com

Media
ICR Westwicke
Ben Shannon, 443-213-0495
ben.shannon@westwicke.com

Source: Ocular Therapeutix, Inc.