Ocular TherapeutixTM Reports on Topline Results of Phase 2b Glaucoma Clinical Trial

October 22, 2015

Clinically meaningful intraocular pressure reduction and improvement in depot retention demonstrated with OTX-TP at day 75

Timolol arm performed better than expected, performance may be improved by placebo plug

No hyperemia-related adverse events observed in any of the patients treated with OTX-TP

Conference call today at 5:00 pm Eastern Time

BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 22, 2015-- Ocular Therapeutix, Inc. (NASDAQ:OCUL), a biopharmaceutical company focused on the development and commercialization of innovative therapies for diseases and conditions of the eye, today announced topline results through 90 days of therapy from its Phase 2b clinical trial of OTX-TP (sustained release travoprost) for the treatment of glaucoma and ocular hypertension.

In this trial, the duration of effect as measured by the clinically meaningful reduction of intraocular pressure (IOP) in the 4.5-5.7 mmHg range was observed out to 90 days with the sustained release OTX-TP drug product. This IOP lowering was comparable to levels seen in the treatment group with the same drug release rate in the Phase 2a clinical trial of OTX-TP. In the 2b trial, the patient group receiving timolol as a comparator drug in the presence of a placebo depot, achieved IOP lowering results that were higher than expected, based on results reported in the peer-reviewed literature. However, as previously reported, the trial was not powered to detect statistically significant differences in IOP lowering between patient groups.

There were no hyperemia-related adverse events noted in any of the patients treated with OTX-TP. Further, there have been no serious adverse events observed to date in the Phase 2b trial, and adverse events were generally similar in frequency across the treatment groups. A complete analysis of all safety data is pending study completion.

“We are pleased with the performance of OTX-TP as seen in the topline results of our Phase 2b clinical trial for the treatment of glaucoma,” stated Amar Sawhney, Ph.D., President, Chief Executive Officer and Chairman of Ocular Therapeutix. “The effect on IOP lowering observed with OTX-TP was similar to what we observed in our Phase 2a study and reached a clinically meaningful level. We also observed a significant improvement in retention of the depot over prior studies through day 75, achieving 88% retention in the combined arms in the study, a testament to the successful modifications to the depot that have been made since the Phase 2a trial. We have advanced our plug designs in parallel with conducting the Phase 2b trial and plan to incorporate the findings from the Phase 2b trial to further optimize our drug product with a higher level of drug release and to achieve balance between retention and patient experience with OTX-TP. Our goal is to advance this program to Phase 3 trial expected in the second half of 2016 with a 75 day drug delivery product after some minor modification of our product.”

Robert Noecker, MD, Ophthalmic Consultants of Connecticut, and Assistant Clinical Professor, Yale University School of Medicine, stated, “Compliance remains a major issue with current glaucoma eye drop therapies. The OTX-TP product candidate addresses this issue directly, and has the potential to remove the burden on the patient to acquire and administer eye drops on a daily basis. In clinical trials to date, OTX-TP has been shown to cause no hyperemia (redness) change from baseline and its preservative-free formulation is benign to the ocular surface. In the current clinical trial, timolol performed better than reported in previous trials most likely due to the use of placebo plugs to keep drug on the surface longer than typical and out of the systemic circulation. OTX-TP is a product candidate that may play an important role in the treatment of patients with glaucoma.”

The prospective, multicenter, randomized, double-masked, double dummy, parallel-arm, active controlled study enrolled 73 patients at 11 clinical sites in the United States to assess the efficacy and safety of OTX-TP as compared to timolol. The study was designed to inform the further clinical development for OTX-TP and was not powered to show statistical significance between study groups. Study efficacy measures included differences in the mean intraocular pressure (IOP) change between the treatment groups from baseline at multiple time points throughout the study. Additional objectives included depot retention, visualization, and replacement if required, duration for washout to achieve stable baseline IOP, as well as a comparison of safety profile. OTX-TP is administered by a physician as an intracanalicular depot through the punctum, a natural opening in the eyelid, and is designed to deliver travoprost to the ocular surface for up to 90 days.

The timolol group was observed to have intraocular pressure lowering of 6.4-7.6 (with an average of 7.0) mmHg compared with baseline, and as noted, this was higher than expected, possibly due to enhanced residence time of the drug on the ocular surface due to occlusion of the punctum by the placebo vehicle depot. The average intraocular pressure lowering seen in four published studies using timolol was 6.2 mmHg. At day 60, which was the primary efficacy measure, the OTX-TP group had an IOP lowering effect of 4.8 mmHg, compared with IOP lowering of 6.4 mmHg for the timolol arm and at day 90, which was a secondary efficacy measure, the OTX-TP group had an IOP lowering effect of 5.2 mmHg, compared with an IOP lowering effect of 7.3 mmHg in the timolol arm.

The Phase 2b interim results are reported on all patients followed through a 90-day duration. Complete results on safety and efficacy through the end of the study are expected to be available in the first quarter of 2016.

Depots were found to be retained in 91% at day 60 and 88% of patients at day 75 when evaluating all patients completing the study through its 90-day duration. Retention was 48% at day 90, reflecting the corresponding absorption and clearance of the depots with the duration of drug release.

All patients were able to visualize the presence of the depots throughout the trial and ask for replacement depots if and when required. All but two OTX-TP treated patients were able to successfully receive a replacement product when their depots were lost any time prior to day 90.

Baseline (washout) evaluations for IOP were conducted at 4 weeks and 5 weeks following screening. It was seen that baseline IOP continued to drop by 1.0 mmHg for the OTX-TP group and 0.61 mmHg for the timolol group from week 4 to week 5, signaling the potential need for longer washout duration in future studies.

The Company plans to investigate in non-significant risk studies whether the presence of the high-retaining placebo depots can be enhancing the effect of timolol, as this has been reported in the literature. The Company also plans to discuss potential clinical study designs with FDA to potentially minimize this effect and better reflect real world usage.

About Glaucoma and Ocular Hypertension

Glaucoma and ocular hypertension are chronic, sight-threatening diseases in which elevated levels of intraocular pressure are associated with damage to the optic nerve, which may result in irreversible vision loss. Glaucoma is the second leading cause of blindness in the world. Ocular hypertension is characterized by elevated levels of intraocular pressure without any optic nerve damage. Patients with ocular hypertension are at high risk of developing glaucoma. In the U.S. alone 2.7 million people suffer from glaucoma. According to IMS, there were 33 million prescriptions and sales of over $2.4 billion of drugs administered by eye drops for the treatment of glaucoma in 2014.

About Sustained Release Travoprost

Sustained Release Travoprost (OTX-TP) is a preservative-free drug product candidate that resides within the canaliculus and delivers the prostaglandin analog travoprost to the ocular surface for up to 90 days. The drug release is designed to deliver a continuous steady release dose is sustained throughout the treatment period. A fluorescent visualization aid is formulated within the product to enable both the physician and the patient to monitor drug presence throughout the course of therapy.

Conference Call & Webcast Information

Members of the Ocular Therapeutix management team will host a live conference call and webcast today at 5:00 pm Eastern Time. The live webcast can be accessed by visiting the investor section of the Company's website at investors.ocutx.com. Please connect at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call 844-464-3934 (U.S.) or 765-507-2620 (International) to listen to the conference call. The conference ID number for the live call will be 66607929. An archive of the webcast will be available until November 5, 2015 on the company's website.

About Ocular Therapeutix, Inc.

Ocular Therapeutix, Inc. (NASDAQ: OCUL) is a biopharmaceutical company focused on the development and commercialization of innovative therapies for diseases and conditions of the eye using its proprietary hydrogel platform technology. Ocular Therapeutix's lead product candidates are in Phase 3 clinical development for post-surgical ocular inflammation and pain and allergic conjunctivitis, and Phase 2 clinical development for glaucoma and inflammatory dry eye disease. The Company is also evaluating sustained-release injectable anti-VEGF drug 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 statements about the development of the Company's product candidates, such as the ongoing development and potential utility of OTX-TP for glaucoma and ocular hypertension and the timing, design and conduct of potential Phase 3 clinical trials of OTX-TP, the Company's plans for regulatory submissions and the advancement of the Company's other product candidates, the potential for the Company's sustained release hydrogel depot technology 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, 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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