Ocular Therapeutix™ Announces Topline Results of Second Phase 3 Clinical Trial of DEXTENZA™ for the Treatment of Allergic Conjunctivitis

June 6, 2016

Primary endpoint for ocular itching not achieved

Conference call today at 8:30 am Eastern Time

BEDFORD, Mass.--(BUSINESS WIRE)--Jun. 6, 2016-- 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 from its second Phase 3 clinical trial to evaluate the safety and efficacy of DEXTENZA™ (sustained release dexamethasone) Intracanalicular Depot for the treatment of ocular itching associated with chronic allergic conjunctivitis. DEXTENZA is a product candidate administered by a physician as a bioresorbable intracanalicular depot and designed for drug release to the ocular surface for up to 30 days.

The single primary endpoint of the trial, defined as the difference in the mean scores in ocular itching between the treatment group and the placebo comparator group at three time points 7 days following insertion of the depots, was not achieved. While mean ocular itching was seen to be numerically lower (more favorable) in the DEXTENZA treatment group compared to the placebo group measured 7 days following insertion of the depots, at 3, 5, and 7 minutes by -0.18, -0.29, and -0.29 units, respectively, on a five point scale, this difference did not reach statistical significance. In addition, the trial did not achieve the requirement of at least a 0.5 unit difference at all three time points 7 days following insertion of the depots and at least a 1.0 unit difference at the majority of the three time points between the treatment group and the placebo group 7 days following insertion of the depots.

The trial also assessed conjunctival redness as a secondary endpoint. The differences in the mean scores in conjunctival redness between the DEXTENZA treatment group and the placebo group 7 days following insertion of the depots at 7, 15 and 20 minutes were -0.35, -0.39 and -0.42, respectively, compared with values of -0.26, -0.32 and -0.41, respectively, at the same time points 7 days following insertion of the depots in the first Phase 3 trial.

The results from the second Phase 3 trial contrast with those achieved in the first Phase 3 clinical trial of DEXTENZA for the treatment of allergic conjunctivitis announced in October 2015, in which the primary endpoint of treatment of ocular itching associated with allergic conjunctivitis was successfully achieved, with mean ocular itching scores being lower in the DEXTENZA group at 3, 5, and 7 minutes by -1.02, -0.87, and -1.04 units, respectively ($p<0.0001$), 7 days following insertion of the depots.

In the second Phase 3 clinical trial, as well as other DEXTENZA clinical trials completed to date regardless of indication, DEXTENZA has exhibited a strong safety profile and has been generally well-tolerated. There were no serious adverse events observed in the second Phase 3 clinical trial.

“We are disappointed that the primary endpoint of ocular itching associated with allergic conjunctivitis was not achieved in our second Phase 3 trial in these patients, and this result is inconsistent with what we saw in our first Phase 3 trial,” said Amar Sawhney, Ph.D., President, Chief Executive Officer and Chairman. “We are currently in the process of conducting a thorough analysis of the data from the second Phase 3 trial to fully understand the difference in efficacy between the two Phase 3 trials. There was a greater variability in ocular itching exhibited by patients in the second Phase 3 trial over the multiple allergen challenges 7, 14 and 28 days following insertion of the depots, compared to the first Phase 3 trial. In a post hoc analysis, when ocular itching scores were averaged over these multiple visits, a statistically significant reduction of symptoms over the entire 1 month intended duration of sustained release, single dose therapy was observed in the DEXTENZA treatment group relative to the placebo vehicle group. Since this analysis was not part of the original end points, we plan to meet with the FDA to discuss the results and chart an appropriate path forward for the development of DEXTENZA for the treatment of allergic conjunctivitis.”

Dr. Sawhney continued, “We remain confident in the potential of our innovative sustained release platform to address diverse applications in ophthalmology. We look forward to the July 2016 PDUFA date for DEXTENZA for the treatment of post-surgical ocular pain.”

Second Phase 3 Study Design

This Phase 3 prospective, U.S.-based multicenter, 1:1 randomized, double-masked, vehicle-controlled trial in 86 patients was designed to evaluate the safety and efficacy of DEXTENZA™ (sustained release dexamethasone), Intracanalicular Depot for the treatment of signs and symptoms of chronic allergic conjunctivitis. This was the second Phase 3 trial which evaluated DEXTENZA versus a placebo vehicle punctum plug using Ophthalmic Research Associate’s modified Conjunctival Allergen Challenge (Ora-CAC ®) Model (Ora, Inc., Andover, MA), which accommodates for the longer therapeutic effect of a seasonal one-time administered drug product.

The trial was designed to assess the effect of DEXTENZA compared with placebo on allergic reactions. DEXTENZA or placebo was administered 48 to 72 hours after the first set of allergen challenges confirming the appropriate allergen and dose exposure to incite an allergic response, followed by three series of successive allergen challenges over a 30 day period. The primary efficacy endpoint evaluated was the difference in mean scores in ocular itching between the treatment group and the placebo vehicle group at three time points 7 days following insertion of the depots. The trial also included a secondary efficacy endpoint for conjunctival redness which evaluated the difference in mean scores in conjunctival redness between the treatment group and the placebo vehicle group at three time points 7 days following insertion of the depots.

Ocular Therapeutix reported topline results of its first Phase 3 allergic conjunctivitis clinical trial with DEXTENZA in October 2015, where the primary endpoint for ocular itching associated with allergic conjunctivitis was successfully achieved and the primary endpoint for conjunctival redness was not achieved.

About Allergic Conjunctivitis

Allergic conjunctivitis is an inflammatory disease of the conjunctiva resulting primarily from a reaction to allergy-causing substances such as pollen or pet dander. The primary symptom of this inflammation is acute ocular itching and the primary sign is conjunctival redness. Allergic conjunctivitis ranges in clinical severity from relatively mild, common forms to more severe forms that can cause impaired vision. According to a study on the management of seasonal allergic conjunctivitis published in 2012 in the peer-reviewed journal Acta Ophthalmologica, allergic conjunctivitis affects 15% to 40% of the U.S. population. For patients with chronic or more severe forms of allergic conjunctivitis, antihistamines and mast cell stabilizers are often not sufficient to treat their signs and symptoms. Many ocular allergy sufferers are not responsive to the conventional dual-acting antihistamine/mast cell stabilizers. These refractory patients are frequently treated with topical corticosteroids administered by eye drops.

About the Modified Conjunctival Allergen Challenge (Ora-CAC®) Model

The modified Ora-CAC® model used in the recently completed clinical trial has been developed to study the interactions between the early and late phases of the allergic response in the eye, and to evaluate the effects of pharmaceutical intervention. The modified Ora-CAC® model utilizes four challenges conducted over a 2-day interval to evaluate the effectiveness of a test agent to prevent an acute ocular allergic reaction, as well as evaluate the test agent's ability to prevent an acute ocular allergic reaction in the presence of subclinical late phase inflammation.

Conference Call & Webcast Information

Members of the Ocular Therapeutix management team will host a live conference call and webcast today at 8:30 am Eastern Time to discuss these results.

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 27710660. An archive of the webcast will be available until June 20, 2016 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 candidate, DEXTENZA™ (sustained release dexamethasone) Intracanalicular Depot, is in Phase 3 clinical development for post-surgical ocular inflammation and pain and allergic conjunctivitis, and in Phase 2 clinical development for dry eye disease. A New Drug Application (NDA) for the post-operative ocular pain indication has been filed with FDA and has a Prescription Drug User Fee Act (PDUFA) target action date of July 24, 2016. A third Phase 3 clinical trial is being conducted for post-surgical ocular inflammation and pain. For glaucoma and ocular hypertension, the Company has completed its End-of-Phase 2 review with the FDA, and the first of two planned OTX-TP (sustained release travoprost) Phase 3 clinical trials is expected to be initiated in the third quarter of 2016. Ocular Therapeutix 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 and regulatory status of the Company's product candidates, such as the Company's expectations and plans regarding regulatory submissions for and the timing and conduct of clinical trials of DEXTENZA™ for the treatment of allergic conjunctivitis, DEXTENZA for post-surgical ocular inflammation and pain, including our expectations regarding the pending PDUFA date for the NDA filed with the FDA, DEXTENZA for dry eye disease and OTX-TP for the treatment of glaucoma and ocular hypertension, the ongoing development of the Company's sustained release hydrogel depot technology and the advancement of the Company's other product candidates, the potential utility of any of the Company's product candidates, 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 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 or any product candidate that receives regulatory approval, 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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