Ocular Therapeutix™ Announces Encouraging Top-Line Results from Exploratory Phase 2 Trial for DEXTENZA™ in Patients with Inflammatory Dry Eye Disease

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- ***DEXTENZA showed benefit in efficacy measure of corneal staining signs of inflammatory dry eye disease for total corneal staining change from baseline at day 30***
- ***Supportive measures of conjunctival staining also showed clinically meaningful benefit***
- ***Improvement of frequency and severity of symptoms of eye dryness, itchiness, and scratchiness also seen***

BEDFORD, Mass.--(BUSINESS WIRE)--Dec. 14, 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 from a Phase 2 exploratory clinical trial designed to evaluate a range of objective and subjective measures (signs and symptoms, respectively) for DEXTENZA™ (sustained release dexamethasone) Intracanalicular Depot for the treatment of inflammatory dry eye disease. This trial was intended to explore which measures would be appropriate to include in the design of a future clinical trial to evaluate efficacy of DEXTENZA or other molecules in a sustained release product as a potential therapy for dry eye disease.

The Phase 2 exploratory clinical trial was a prospective, multicenter, randomized, parallel-arm, bilateral, double-masked, vehicle controlled study to explore the safety and efficacy of DEXTENZA for the treatment of subjects exhibiting signs and symptoms of moderate to severe dry eye disease. The trial was conducted at two sites in the United States that included 43 patients (86 eyes) and was not powered for statistical significance. Designed as a serial phase study, patients were initially administered a placebo vehicle depot for 45 days to establish a baseline for the investigational drug treatment. Patients who responded to the placebo vehicle depot, i.e., showed benefit from punctal occlusion alone, were excluded from the treatment phase of the study. Qualifying patients who continued to exhibit signs and symptoms of dry eye during the initial 45 days were enrolled in the treatment phase of 30 days, randomized to receive either DEXTENZA or a placebo vehicle in a 1:1 ratio.

In this trial, patients were selected for a minimum threshold of signs of corneal staining and were randomized to either treatment with DEXTENZA or a placebo vehicle depot. Patients were stratified into groups based on the level of National Eye Institute aggregate corneal fluorescein staining score improvement and were then randomized into the treatment or placebo vehicle depot group per a pre-determined randomization list to maintain masking. DEXTENZA treated patients showed clinically meaningful benefits compared to patients receiving a placebo vehicle depot, with improvement in total and inferior corneal staining as well as conjunctival staining. While the study was not designed to show statistical significance, total corneal staining at day 30 following randomization was significantly decreased from baseline in the DEXTENZA group (-3.14) compared to placebo (-1.10) (p=0.018). Inferior staining showed clinically significant differences in the change from baseline in the DEXTENZA treatment group compared to placebo (-0.43 and -0.45 at Day 15 and Day 30 respectively). Corneal staining is a primary endpoint that has been used in recent phase 3 dry eye clinical trials conducted by other ophthalmology companies for dry eye disease. Supportive analyses of lissamine green staining also demonstrated a clinically significant change in favor of DEXTENZA, where total staining was more than 1 point improved for the DEXTENZA group compared to the placebo.

While patients in this trial did not have a specific inclusion threshold for symptoms of dry eye disease at the time of randomization, changes from baseline to Day 15 and Day 30 in frequency and severity of symptoms of eye dryness, itchiness, and scratchiness, as measured by the standard patient evaluation of eye dryness (SPEED) questionnaires were noted to favor the patient groups treated with DEXTENZA. There were certain other measures included in the trial where DEXTENZA did not show a benefit over the placebo group. The trial was designed to include many different measures and identify those measures where DEXTENZA showed a benefit compared with the placebo vehicle depot group to inform the design of future clinical trials. The trial achieved this objective.

“We are encouraged that DEXTENZA showed improvements in the important and highly relevant efficacy measures of total and inferior corneal staining in this exploratory study. It is apparent that DEXTENZA exhibited a global effect across all regions on the cornea, particularly the inferior region, which is generally considered to be the most susceptible region of the cornea for the onset of dry eye disease,” said Amar Sawhney, Ph.D., President, Chief Executive Officer and Chairman of Ocular Therapeutix, Inc. “We intend to advance this important clinical program to expand DEXTENZA’s potential for this highly unmet need. Inflammatory dry eye is one of the most common ophthalmic disorders affecting approximately 20 million people in the United States, for which novel treatments are needed. With the results from this exploratory trial, we are now positioned to pursue the further development of DEXTENZA for inflammatory dry eye disease.”

"Subject to further clinical testing, DEXTENZA may be able to satisfy an unmet need for our patients with dry eye disease with its dual mechanism of action, preservative free formulation, and rapid onset efficacy with minimal compliance or safety concerns," stated John Sheppard, M.D., M.M.Sc., Principal Investigator, Virginia Eye Consultants (Norfolk, VA) and Professor of Ophthalmology, Eastern Virginia Medical School (Norfolk, VA).

This initial exploratory dry eye study has helped to identify and concentrate the optimal patient population for sign responders. Additional work will be done to identify and concentrate the proper study population for symptoms responders.

While safety data continues to be analyzed, overall DEXTENZA had a good safety profile and was well tolerated. There were no intraocular pressure spikes noted in the trial. The retention rate of the drug depots was 96% through day 30 of the treatment phase of the trial.

About DEXTENZA

DEXTENZA is a product candidate administered by a physician as a bioresorbable intracanalicular depot and designed for extended drug release to the ocular surface for 30 days. Ocular Therapeutix is developing DEXTENZA for a broad range of front of the eye conditions. In September 2015, the

Company filed an NDA with the Food and Drug Administration (FDA) for DEXTENZA for the treatment of post-surgical ocular pain and a PDUFA date of July 24, 2016 has been set by the FDA. The Company is conducting a third Phase 3 clinical trial to potentially broaden its indication to include post-surgical ocular inflammation and pain. After a successful initial Phase 3 study for the treatment of allergic conjunctivitis, a second Phase 3 clinical trial is currently enrolling for DEXTENZA for this additional indication.

About Dry Eye Disease

Dry eye disease affects the ocular surface and is characterized by dryness, inflammation, pain, discomfort, and irritation. Dry eye is a complex, multifactorial disease which can present differently in patients, and becomes more common with age. Due to the prevalence of the disease, over $1.5 billion was spent on treatment of the disease in the United States alone in 2014 in both prescription and artificial tear products (IMS Health). One cause of the disease is inflammation of the ocular surface resulting from a patient's immune response. Although physicians may prescribe topical steroid eye drops for the treatment of dry eye disease, chronic use of topical steroids can lead to elevations in intraocular pressure, which is a risk factor for glaucoma. DEXTENZA only contains approximately 7% of the amount of active dexamethasone compared with eye drop therapy, resulting in an improved safety profile. Conversely, patients often do not reliably self-administer these drops, which can lead to eye irritation and continual, if not more, inflammation.

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™, is in Phase 3 clinical development for post-surgical ocular inflammation and pain and allergic conjunctivitis, and in Phase 2 clinical development for inflammatory dry eye disease. An NDA for the post-operative ocular pain indication has been submitted to the FDA and a third Phase 3 clinical trial is being conducted for post-operative ocular inflammation and pain. The Company's product candidate, OTX-TP (sustained release travoprost) intracanalicular depot, has completed a Phase 2b clinical trial for glaucoma and ocular hypertension. 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 or potential commercialization of the Company's product candidates, such as the Company's clinical development of DEXTENZA for the treatment of inflammatory dry eye disease, the Company's plans and expectations regarding regulatory submissions and the design and conduct of a third Phase 3 clinical trial of DEXTENZA™ for post-surgical inflammation and pain, the timing and conduct of a second Phase 3 clinical trial of DEXTENZA for the treatment of allergic conjunctivitis, the timing and conduct of the Company's additional development work and clinical trials of OTX-TP for the treatment of glaucoma and ocular hypertension and the ongoing development of the Company's sustained release hydrogel depot technology, the advancement of the Company's other product candidates, the potential utility of any of the Company's product candidates 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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