Ocular Therapeutix™ Announces Topline Phase 1 Clinical Trial Results of OTX-CSI

October 8, 2020

OTX-CSI demonstrates improvement in signs and symptoms of Dry Eye Disease

BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 8, 2020-- 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 topline Phase 1 clinical trial results of OTX-CSI (cyclosporine intracanalicular insert) for the treatment of dry eye disease (DED).

“The results of the Phase 1 clinical trial provide an early look at the safety, tolerability, durability, and potential biological activity of OTX-CSI,” said Michael Goldstein, MD, MBA, Chief Medical Officer. “While the study enrolled a small number of subjects and was open-label, the results seen thus far are very encouraging. Our novel intracanalicular insert delivered a consistent dose of cyclosporine without preservatives over approximately 12 weeks, in a manner we believe to be both less irritating to the ocular surface and also faster acting than current standard of care eye drop therapies. We believe that if approved by the FDA, OTX-CSI has the potential to become a highly differentiated treatment that would provide significant benefit to patients with dry eye disease and allow them a hands-free alternative to current therapies.”

The Phase 1, U.S.-based, open label, single-center clinical trial was intended to evaluate safety, tolerability, durability, and biological activity of OTX-CSI by measuring signs and symptoms of DED in five subjects (10 eyes) over approximately 16 weeks (a 12-week study period, with an additional 4-week safety follow-up). In the clinical trial, OTX-CSI was administered by a physician as a bioresorbable intracanalicular insert to both eyes in five subjects with DED. All subjects completed the 16-week study period with no drop-outs. No serious adverse effects were reported. The inserts were observed to be well-tolerated, and there were no adverse events of stinging, irritation, blurred vision or tearing reported or observed. Tear production as measured by the Schirmer’s test improved from mean values of 4.2 mm at baseline to 8.2 mm at Week 12. One of five (20%) subjects had a ≥ 10mm increase in Schirmer’s score at Week 12 from baseline. Subjects treated with OTX-CSI demonstrated an improvement in signs of DED as measured by corneal total fluorescein staining (a mean value of 6.7 at baseline, improved to a mean value of 2.7 at Week 12, on a scale of 0 to 15) and an improvement in symptoms of DED as measured by the visual analog scale (VAS) eye dryness severity score (a mean value of 51 at baseline, improved to a mean value of 33 at Week 12, on a scale of 0 to 100) and the VAS dry eye frequency score (a mean value of 51 at baseline, improved to a mean value of 31 at Week 12, on a scale of 0 to 100). The onset of action of OTX-CSI was seen as early as two weeks for both signs and symptoms of DED and continued over the 16 week study period.

The Company recently announced that the first patient was dosed in a Phase 2, U.S.-based, randomized, masked, vehicle controlled, multi-center clinical trial evaluating two different formulations of OTX-CSI with vehicle insert in approximately 105 subjects who will be followed for a period of 16 weeks.

About Dry Eye Disease

Dry eye disease is a common, multifactorial disease of the tears and ocular surface that results in symptoms of discomfort (such as burning sensation, itching, redness, stinging, pain and foreign body sensation), visual disturbance, and tear film instability that can cause potential damage to the ocular surface. Inflammation of the lacrimal gland and ocular surface have been shown to play a key role in dry eye disease, resulting in a reduction in tear production. Dry eye disease is one of the most common ophthalmic disorders presenting to clinicians and the Market Scope 2019 Dry Eye Products Market Report estimated that more than 17.2 million adults in the United States have been diagnosed with the disorder, including an estimated 8.6 million classified as having a moderate to severe form of the disease.

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 FDA-approved for the treatment of ocular inflammation and pain following ophthalmic surgery. Ocular Therapeutix recently completed a Phase 3 clinical trial evaluating DEXTENZA for the treatment of ocular itching associated with allergic conjunctivitis. The Company’s earlier stage development assets currently in Phase 1 trials include OTX-TIC (travoprost intracameral implant) for the reduction of intraocular pressure in patients with primary open-angle glaucoma and ocular hypertension, OTX-CSI (cyclosporine intracanalicular insert) for the treatment of the signs and symptoms of dry eye disease and OTX-TKI (axitinib intravitreal implant) for the treatment of retinal diseases. Also, Ocular Therapeutix is currently developing OTX-DED (dexamethasone intracanalicular insert) for the treatment of episodic dry eye and, in collaboration with Regeneron, OTX-AFS (aflibercept suprachoroidal injection) for an extended-delivery formulation of aflibercept for the treatment of retinal diseases, and 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 the commercialization of DEXTENZA®, ReSure® Sealant, or any of the Company’s product candidates; the commercial launch of, and effectiveness of reimbursement codes for, DEXTENZA; the conduct of post-approval studies of DEXTENZA; the development and regulatory status of the Company’s product candidates, such as the Company’s development of and prospects for approvability of DEXTENZA for additional indications including allergic conjunctivitis, OTX-DED for the treatment of episodic dry eye disease, OTX-CSI for the treatment of dry eye disease, OTX-TIC for the treatment of primary open-angle glaucoma and ocular hypertension, OTX-TKI for the treatment of retinal diseases including wet AMD, and OTX-AFS as an extended-delivery formulation of the VEGF trap aflibercept for the treatment of retinal diseases including wet AMD; the ongoing development of the Company’s extended-delivery hydrogel depot technology; the size of potential markets for our product candidates; the potential utility of any of the Company’s product candidates; the potential benefits and future operation of the collaboration with Regeneron Pharmaceuticals, including any potential future payments thereunder; projected net product revenue and other financial metrics of DEXTENZA; the expected impact of the COVID-19 pandemic on

the Company and its operations; 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, the timing and costs involved in commercializing DEXTENZA, ReSure Sealant or any product candidate that receives regulatory approval, including the conduct of post-approval studies, the ability to retain regulatory approval of DEXTENZA, ReSure Sealant or any product candidate that receives regulatory approval, the ability to maintain reimbursement codes for DEXTENZA, the initiation, timing 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 Company's ability to generate its projected net product revenue on the timeline expected, if at all, the sufficiency of cash resources, the Company's existing indebtedness, the ability of the Company's creditors to accelerate the maturity of such indebtedness upon the occurrence of certain events of default, the outcome of the Company's ongoing legal proceedings, the severity and duration of the COVID-19 pandemic including its effect on the Company's and relevant regulatory authorities' operations, the 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 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 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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Investors
Ocular Therapeutix
Donald Notman
Chief Financial Officer
dnotman@ocutx.com

or

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

Media
Ocular Therapeutix
Scott Corning
Senior Vice President, Commercial
scorning@ocutx.com

Source: Ocular Therapeutix, Inc.