Ocular Therapeutix™ Announces Topline Results for Phase 2 Clinical Trial of OTX-CSI for the Treatment of Dry Eye Disease Did Not Meet Primary Endpoint

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BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 22, 2021-- 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 results from its Phase 2 clinical trial of OTX-CSI (cyclosporine intracanalicular insert) for the treatment of dry eye disease (DED).

The Phase 2, U.S.-based, randomized, double-masked, multi-center, vehicle-controlled clinical trial of OTX-CSI was designed to evaluate safety, tolerability, durability, and efficacy of two different formulations of OTX-CSI by measuring signs and symptoms of DED in 140 subjects treated in both eyes over approximately 16 weeks (a 12-week study period, with an additional 4-week safety follow-up). In the Phase 2 clinical trial, OTX-CSI was administered to 147 subjects with DED at 15 sites in the U.S. The four groups evaluated in this study were: OTX-CSI for a shorter duration (2-3 months formulation-F1, n=42), OTX-CSI for a longer duration (3-4 months formulation-F2a, n=40), vehicle insert for a longer duration (3-4 months formulation-F2b, n=43) and vehicle insert for a very short duration (1 week formulation-F3, n=22).

The study did not show separation between the OTX-CSI treated subjects (both formulations) and the vehicle treated subjects (both formulations) for the primary endpoint of increased tear production at 12 weeks as measured by the Schirmer's Test. Change from baseline (improvement) in mean Schirmer's Test scores for the four groups were as follows: OTX-CSI F1: 1.98 mm, OTX-CSI F2a: 1.91 mm, Vehicle F2b: 2.24 mm and Vehicle F3: 3.08 mm.

The study did show an improvement compared with baseline in signs of dry eye disease as measured by total corneal fluorescein staining (CFS) and symptoms of dry eye disease as measured by the visual analogue scale (VAS) eye dryness in subjects treated with the OTX-CSI insert (both formulations) starting as early as two weeks after insertion and continuing over the 12 weeks study period. These improvements were not statistically significant compared with vehicle insert (both formulations) for either CFS or VAS eye dryness (severity and frequency) at 12 weeks.

Overall, the OTX-CSI insert (both formulations) was generally observed to have a favorable safety profile and was well tolerated. There were no ocular serious adverse events. No subjects dropped out of the trial due to an adverse event. The most common ocular adverse event was ocular pruritis which was seen in less than 16% of subjects. The adverse events of ocular discomfort or pain were seen in less than 3% of subjects. The most common non-ocular event was COVID-19 and was seen in 3% of subjects.

"We would like to thank the patients and investigators who participated in the OTX-CSI clinical trial," said Antony Mattessich, President and Chief Executive Officer of Ocular Therapeutix. "While we are disappointed by these results, demonstrating clinical benefit in patients with dry eye disease remains a significant unmet need and we will continue to review the data for additional information that may inform future development of this program. We remain confident in the potential of our hydrogel-based formulation technology and its ability to deliver innovative ophthalmology therapies. We look forward to our anticipated Phase 2 top-line read out for OTX-DED for the short-term treatment of signs and symptoms of dry eye disease. In addition, we expect to provide updates on other pipeline programs being developed to treat glaucoma and wet-AMD as well as updates on our currently marketed product, DEXTENZA®, which recently received FDA approval to expand its label for the treatment of ocular itching associated with allergic conjunctivitis."

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 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 currently in Phase 1 clinical trials include OTX-TKI (axitinib intravitreal implant) for the treatment of wet AMD and other retinal diseases and OTX-TIC (travoprost intracameral implant) for the reduction of intraocular pressure in patients with primary open-angle glaucoma or ocular hypertension. In addition to OTX-CSI (cyclosporine intracanalicular insert) for the chronic treatment of dry eye disease, Ocular Therapeutix is currently evaluating OTX-DED (dexamethasone intracanalicular insert) for the short-term treatment of the signs and symptoms of dry eye disease in Phase 2 clinical trials. Ocular Therapeutix's first product, ReSure® Sealant, is an FDA-approved device to prevent wound leaks in corneal incisions following cataract surgery.

This release discusses investigational agents in development and no conclusions can or should be drawn relating to the efficacy or safety of these agents. There is no guarantee that any investigational agents will successfully complete clinical development or gain FDA approval.

About DEXTENZA

DEXTENZA is FDA approved for the treatment of ocular inflammation and pain following ophthalmic surgery and ocular itching associated with allergic conjunctivitis. DEXTENZA is a corticosteroid intracanalicular insert placed in the punctum, a natural opening in the inner portion of the lower eyelid, and into the canaliculus and is designed to deliver dexamethasone to the ocular surface for up to 30 days without preservatives. DEXTENZA resorbs and exits the nasolacrimal system without the need for removal.

Please see full Prescribing and Safety Information at www. DEXTENZA.com.

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 and compliance with related labeling requirements for DEXTENZA and ReSure Sealant; the development and regulatory status of the Company's product candidates, such as the Company's development of and prospects for approvability of OTX-CSI for the chronic treatment of dry eye disease, OTX-DED for the short-term treatment of the signs and symptoms of dry eye disease, OTX-TIC for the treatment of primary open-angle glaucoma or ocular hypertension, and OTX-TKI 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 operations of Company collaborations, including any potential future costs or payments thereunder; projected net product revenue, in-market sales and other financial and operational metrics of DEXTENZA and ReSure Sealant; potential market sizes for indications targeted by the Company's product candidates, if approved; 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 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, ReSure Sealant or any product candidate that receives regulatory approval, including the conduct of post-approval studies, the ability to successfully develop and commercialize products for the ophthalmology office setting, the ability to retain regulatory approval of DEXTENZA, ReSure Sealant or any 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, timing, conduct and outcomes of clinical trials, availability of data from clinical trials and expectations for regulatory submissions and approvals, the interpretation of data from the Company's clinical trials, the Company's ability to enter into and perform its obligations under collaborations and the performance of its collaborators under such collaborations, 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 and in-market sales 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 severity and duration of the COVID-19 pandemic including its effect on the Company's and relevant regulatory authorities' operations, any additional financing needs 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.

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