Ocular Therapeutix™ Receives FDA Agreement Under Special Protocol Assessment (SPA) for Registrational Trial of AXPAXLI™ in NPDR

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Ocular plans to outline clinical trial design, timing, and development strategy for non-proliferative diabetic retinopathy (NPDR) and diabetic macular edema (DME) at its Investor Day on September 30, 2025

BEDFORD, Mass., Aug. 12, 2025 (GLOBE NEWSWIRE) -- Ocular Therapeutix, Inc. (NASDAQ: OCUL, "Ocular"), an integrated biopharmaceutical company committed to redefining the retina experience, today announced it has received written agreement regarding a registrational trial design from the U.S. Food and Drug Administration (FDA) under a Special Protocol Assessment (SPA) for the Company's planned clinical trial of AXPAXLI (also known as OTX-TKI) for the treatment of non-proliferative diabetic retinopathy (NPDR).

"Securing a Special Protocol Assessment agreement from the FDA for our planned NPDR trial represents a major milestone as we look to broaden the impact of AXPAXLI beyond wet AMD and into diabetic eye disease. This formal agreement ensures direct FDA alignment with our proposed approach for NPDR and provides us with a clear regulatory path forward. We are confident AXPAXLI will be successful in the trial design and primary endpoint agreed upon with the FDA," said **Pravin U. Dugel, MD, Executive Chairman, President and Chief Executive Officer** of Ocular Therapeutix. "The opportunity in diabetic eye disease is tremendous, with diabetic retinopathy affecting nearly 9 million people in the U.S. alone. We believe AXPAXLI, with its potential for annual dosing, could offer a transformative approach to improving outcomes while preventing or substantially reducing the rate of vision-threatening complications in these patients. We look forward to providing more details about our strategy in NPDR and DME at our upcoming Investor Day on September 30th."

"A safe, effective, annual intravitreal treatment that aligns with current retina practice dynamics would be a very appealing therapeutic option for people with diabetic retinopathy," commented **Daniel F. Martin, MD, Vice Chair for Clinical Affairs and Professor of Ophthalmology at the Emory University School of Medicine.** "Diabetic retinopathy remains the leading cause of vision loss among working-age adults. In the Phase 1 HELIOS trial, with a single AXPAXLI injection, there were no NPDR patients who had disease progression or vision threatening complications at 48 weeks, while 25% of patients in the sham control arm experienced worsening disease and nearly 40% developed a vision threatening complication. In addition, there was improvement in all patients treated with AXPAXLI who came into the study with non-center involved DME. Though these data are early, they point to the possibility of not only improving outcomes but also reshaping how physicians approach proactive care in NPDR with and without DME."

"At least half of all diabetic patients are expected to develop some form of diabetic retinopathy in their lifetime," added **David M. Brown, MD, Chief Medical Officer of Retina Consultants of America (RCA)**. "With 6.4 million Americans currently living with NPDR alone, the disease burden is staggering, yet fewer than 1% of these patients are treated. This unfortunate dynamic is despite the proven efficacy of anti-VEGFs and is driven primarily by the challenges of ongoing intraocular injections in an asymptomatic but high-risk population. A long-acting, sustainable treatment like AXPAXLI, potentially controlling diabetic retinopathy with one injection per year, could shift the paradigm from reactive treatment to proactive disease management. The potential public health impact of a safe, durable, effective, once-a-year therapy is tremendous as it would decrease the blinding complications from diabetes that we see every day in our clinics."

At its Investor Day, Ocular Therapeutix plans to review its strategy and the next steps for AXPAXLI in NPDR and DME. The event will be held in New York City on September 30, 2025. To sign up for the event, please visit Ocular's website or register HERE.

About AXPAXLI
AXPAXLI™ (also known as OTX-TKI) is an investigational, bioresorbable, intravitreal hydrogel incorporating axitinib, a small molecule, multi-target, tyrosine kinase inhibitor with anti-angiogenic properties, being evaluated for the treatment of wet AMD, diabetic retinopathy, diabetic macular edema, and other retinal diseases.

About Diabetic Eye Disease
Diabetic eye disease is an increasingly prevalent global health concern, driven by the rapidly rising number of individuals diagnosed with diabetes each year.

Diabetic retinopathy (DR) is the most common category of retinal diseases, affecting over an estimated 150 million people worldwide (2024 Market Scope® Retinal Pharmaceuticals Market Report). DR is a progressive condition in which retinal blood vessels are damaged following a cascade of events triggered by chronically elevated levels of blood glucose. As many as half of

all diabetic patients are expected to develop some form of DR in their lifetime. It can progress from non-proliferative (NPDR) stages to proliferative stages (PDR). Fewer than 1% of the 6.4 million NPDR patients in the U.S. receive treatment today, despite the availability of anti-VEGF therapies approved for the indication, largely due to the burden of frequent injections.

Diabetic macular edema (DME) is also a leading cause of vision loss in the working-age population. DME, the result of an accumulation of fluid in the macula that can afflict patients with diabetes, can occur at any stage of DR. In patients with DME, blood vessels in the eyes leak and start to swell, which can cause vision loss or blindness.

About Ocular Therapeutix, Inc.
Ocular Therapeutix, Inc. is an integrated biopharmaceutical company committed to redefining the retina experience. AXPAXLI™ (also known as OTX-TKI), Ocular's investigational product candidate for retinal disease, is an axitinib intravitreal hydrogel based on its ELUTYX™ proprietary bioresorbable hydrogel-based formulation technology. AXPAXLI is currently in Phase 3 clinical trials for wet age-related macular degeneration (wet AMD).

Ocular's pipeline also leverages the ELUTYX technology in its commercial product DEXTENZA ®, an FDA-approved corticosteroid for the treatment of ocular inflammation and pain following ophthalmic surgery in adults and pediatric patients and ocular itching associated with allergic conjunctivitis in adults and pediatric patients aged two years or older, and in its investigational product candidate OTX-TIC, which is a travoprost intracameral hydrogel that is currently in a Phase 2 clinical trial for the treatment of open-angle glaucoma or ocular hypertension.

Explore the Company's new corporate branding and follow the Company on its website, LinkedIn, or X.

Forward-Looking Statements
Any statements in this press release about future expectations, plans, and prospects for the Company, including the commercialization of DEXTENZA; the development, regulatory status of and regulatory submissions regarding the Company's product candidates; the design of the Company's planned clinical trial of AXPAXLI (also known as OTX-TKI) for the treatment of NPDR and DME; the potential utility or adoption, if approved, of any of the Company's product candidates; and other statements containing the words "anticipate", "believe", "estimate", "expect", "intend", "designed", "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 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 any product or product candidate that receives regulatory approval; the ability to retain regulatory approval of any product or product candidate that receives regulatory approval; the ability to maintain and the sufficiency of product, procedure and any other reimbursement codes for any of the Company's approved products; the initiation, design, timing, conduct and outcomes of ongoing and planned clinical trials; the risk that the FDA will not agree with the Company's interpretation of the written agreement under its Special Protocol Assessments for AXPAXLI, including for SOL-1 and for the Company's planned NPDR clinical trial; the risk that the FDA may not agree that the protocol and statistical analysis plan of SOL-R or that the data generated by the SOL-1 and SOL-R trials support marketing approval, even if the trials are successful; the risk that the FDA may not agree that the data generated by the Company's planned clinical program for AXPAXLI in NPDR and DME supports marketing approval in NPDR or DME, even if clinical trials are successful; the risk that the Company and the FDA may not agree on the registrational pathway for any of its product candidates; uncertainty as to whether the data from earlier clinical trials will be predictive of the data of later clinical trials, particularly later clinical trials that have a different design or utilize a different formulation than the earlier trials, whether preliminary or interim data from a clinical trial (including masked safety or masked rescue data from the Company's SOL-1 trial) will be predictive of final data from such trial, or whether data from a clinical trial assessing a product candidate for one indication will be predictive of results in other indications; uncertainty as to whether data from the Company's planned long-term, open-label extension study in wet AMD will demonstrate clinically meaningful, long-term benefits; uncertainties regarding the potential commercial advantages and/or position of the Company's product candidates; availability of data from clinical trials and expectations for regulatory submissions and approvals; the Company's scientific approach and general development progress; uncertainties inherent in estimating the Company's cash runway, future expenses and other financial results, including its ability to fund future operations, including clinical trials; the Company's existing indebtedness and the ability of the Company's creditors to accelerate the maturity of such indebtedness upon the occurrence of certain events of default; 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 may 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.

Investors & Media
Ocular Therapeutix, Inc.

Bill Slattery
Vice President, Investor Relations
bslattery@ocutx.com